A New Voice in Science

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Patient participation in decision-making on biomedical research

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Motivation of the artist: "The various designs of the human figures symbolize the various characters. Despite that they respect and have genuine interest in each another".

VRIJE UNIVERSITEIT

A New Voice in Science

Patient participation in decision-making on biomedical research

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"The choices we make, not the chances we take, determine our destiny"
(anonymous)

Voor Max en Floris

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PREFACE

A new voice in science; this title obviously refers to the voice of the patient that is heard in decision-making concerning biomedical science increasingly. But it is not only the patient that can be subscribed a new voice in a scientific field. In some way, this thesis itself reflects a new voice as well. Almost twenty years ago I entered the scientific world as a biochemical research technician. But now I have raised my voice within the field of Science and Technology Studies. This change has much to do with the motto of this book.

"The choices you make, not the chances you take, determine our destiny." This anonymous quote was the subscript of a beautiful poster that decorated the wall behind my computer during the years this study was undertaken. On the picture is a small boat on the shore. It is still safely tied but ready to go. Shall it sail out? But what lies ahead? A robust rock-work rises from the smooth surface of the sea. And on the horizon, the vague contours of an unknown landscape become visible. This image to some extent represents the last 10 years of my professional life, which was characterized by important but not always easy choices.

Until 1995 I was working as a research technician within the biotechnological and biomedical fields. I loved my work and my colleagues and lived in a nice house near Rotterdam. Still I knew I needed to change course and to face a new challenge. I decided to resign from my professional life, to move to Amsterdam and to go to 'school' again. That was my first and most radical choice. I left my safe, comfortable life behind in order to meet a new but uncertain future. In the following three years I studied (bio)chemistry. The second crucial choice was taken during my study: I broadened the focus of my study from pure chemistry towards policy and management of science. In addition, I started to study philosophy as well. After finalizing my MSc, I faced a new choice: would I accept a job in the field of science policy or would I apply for a PhD position in the field of Science and Technology Studies? As you will guess, I choose the latter. This choice implied not only a career in science, but

also some more years of financial scarcity and uncertainty about a future position and income. Still, I never regretted the choices I made. Firstly, I found my way in a scientific field of which I feel it suits me very well. It enables me to combine my experiences and interest in biomedical science with my new interest in philosophy. Secondly, my new career in Amsterdam brought me also a new private life: in this city I met and married my dear husband and gave birth to two beautiful, lovely boys, to whom I dedicate this book.

Besides to my personal life, I believe the motto refers to the subject of this thesis as well. In the course of this study, I found out that involving patients in decision-making on biomedical research is all but self-evident. It is also a conscious choice; a choice for leaving familiar and stabilized decision-making routes and practices, while searching for new ways of decision-making that involves patients as partners.

When I started this study in 1998, patient participation in decision-making on biomedical research was a new and uncommon subject; it required a lot of flexibility and pioneering. I usually faced scepticism on the desirability and feasibility of such participation, among both researchers and patients. Even when individuals became enthusiastic about the potential of patient participation in decision-making on biomedical research, their organizational base was not prepared to invest in its implementation. Only in the last phase of my study, when I already acquiesced in the idea of writing a more theoretical and descriptive dissertation, did the opportunity arise to bring patient participation into practice. The Netherlands Asthma Foundation was interested and prepared to invest in a social experiment that entailed the practical application of patient participation in research agenda setting. This social experiment could have covered a thesis itself; it entails many starting points for further research and analysis. All the same, I hope this thesis will inspire people to experiment with and implement patient participation in decision-making on (biomedical) research.

Amsterdam, September 2005

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INTRODUCTION

Once considered esoteric and reserved for a small elite of qualified academics, science is increasingly regarded as a societal endeavour which demands a more democratic, participative approach (e.g. Chopyak and Levesque, 2002b; Fuller, 2000; Nowotny et al., 2001). Today's empowered public is less willing to accept the self-proclaimed autonomy of science and increasingly demands evidence of its legitimacy and accountability. In order to forestall social resistance and to ensure public support, new and controversial scientific developments, in biotechnology and genomics for example, have become the topic of consensus conferences and public debates (e.g. Andersen and Jaeger, 1999; Barns et al., 2000; Guston, 1999; Joss and Durant, 1995; Rowe and Frewer, 2000). At the same time, the growing complexity of society and its problems, resulting – among others – from advancing trends of globalization and scientification, requires new methods of knowledge production. Scientists increasingly aim to capitalize on societal needs and changes in contexts of application, for example by integrating knowledge from different (scientific and societal) sources in a transdisciplinary way (e.g. Chopyak and Levesque, 2002a; Cornwall and Jewkes, 1995; Gibbons et al., 1994; Johnson et al., 2003; Kasemir, Jaeger et al., 2003; Pretty, 1995; Thompson Klein et al., 2001; Van Asselt and Rijkens-Klomp, 2002)

Regarding the growing need for participation, it is imperative to consider precisely which actors should be involved in science-related decision-making. Collins and Evans (2002) have addressed this issue explicitly in their discussion paper "The Third Wave of Science Studies: Studies of Expertise and Experience". They identify a political and a technical dimension within such decision-making processes, stating that political legitimacy requires the involvement of all *stakeholders* (those who

have an interest in, or are affected by, the decision), while technical quality requires that relevant *experts* participate. These 'relevant experts' can be people with academic certifications as well as uncertified persons who have obtained experience-based expertise. Relevant non-certified experts may be potential end-users or beneficiaries of research outcomes, people who might be directly or indirectly affected by the research, people who have obtained certain contextual or experiential knowledge, or even the public at large. Their relevance is determined by the content and context of each particular decision-making process and what potential contribution they can make. Their experience-based expertise may relate to societal needs, norms and values, risk perceptions, interests, the relevance of certain research topics or questions, the desirability and appropriateness of solutions, additional explanations or solutions, etc. This knowledge is often referred to as lay knowledge, local knowledge, or indigenous knowledge (e.g. Agrawal, 1995; Lopez Cerezo and Gonzalez Garcia, 1996; Pinton, 2003; Popay and Williams, 1996; Wynne, 1996).

This dissertation focuses on one specific case of stakeholder participation in science-related decision-making, namely patient participation in decision-making on biomedical research. Biomedical research is one discipline within the broad domain of health research, which comprises all medical, social, and environmental research focusing on health and health care issues. It covers the invention and development of (bio)medical technologies, and thus can be considered the scientific foundation of Western medicine. Patients constitute one of the end-user groups of biomedical research, as beneficiaries of the generated knowledge and technologies within this research.[1] Since end-users (or user configurations[2]) strongly determine the design, dissemination, and use of technologies (cf. Oudshoorn and Pinch, 2003), patients can be considered important *stakeholders* of biomedical research. In addition, patients obtain a specific type of knowledge based on their daily experience of their disease. Several scholars have argued that patients' knowledge and experience can complement that of professionals in decision-making processes (Entwistle et al., 1998; Goodare and Smith, 1995; Nordin, 2000; Popay and Williams, 1996; Telford et al., 2002). This knowledge might also be valuable in the context of biomedical knowledge production, which makes patients potentially relevant (non-certified) *experts* within the biomedical field. Both patients' roles of relevant stakeholders and potential relevant experts

1 'public downstream users' in the categorization of Lyall et al. (2004).

2 as 'co-constructed' with technologies (Oudshoorn and Pinch, 2003)

found arguments in favour of their'[3] active involvement in decision-making on biomedical research. As has been argued by Collins and Evans (2002), involving relevant stakeholders and experts in decision-making may enhance the political legitimacy and the 'technical' quality of this decision-making respectively.

Despite these arguments, a significant lack of academic literature on patient participation in biomedical research suggests that patients are largely uninvolved in decision-making regarding biomedical research agendas. This is in contradiction to the growing reference to patient participation in decision-making processes regarding other types of health research, such as health services research (e.g. Matsunaga et al., 1996; Ong and Hooper, 2003; Phillips and Grams, 2003), health technologies research (e.g. Oliver et al., 2001; Reuzel, 2004), research on public health and prevention (e.g. Bonham and Nathan, 2002; De Koning and Martin, 1996; Israel et al., 1998; Parker et al., 1998), and clinical studies (e.g. Epstein, 1996; Hanley et al., 2001; Kelson, 1999; Koops and Lindley, 2002). Likewise, other scientific fields that aim to address societal problems or needs, such as agricultural research, sustainability research, environmental research, and developmental studies (e.g. Broerse and Bunders, 2000; Johnson et al., 2003; Kasemir, Jäger et al., 2003; Pretty, 1995; Scholz et al., 2000) increasingly rely on societal actors to enrich decision-making with broader perspectives. Since biomedical research aims to contribute to the fundamental health, and therefore quality, of people's lives, and since it demands enormous international financial investment, one would expect that decision-making on this research would involve societal actors as well. Therefore, the apparent lack of patient participation in biomedical research decision-making is surprising and calls for closer scrutiny.

But is it true that patients rarely participate in decision-making on biomedical research, or is this phenomenon just poorly documented? And if it is true, what are the causes of this limited involvement? Do patients themselves abstain from participation? Do biomedical researchers prevent patient participation? Are other obstacles constraining patient participation

[3] In many studies on patient participation in health research, scholars use the term 'consumers', referring to all (potential) users of health care and their representatives (e.g. Hanley et al., 2001; Herxheimer and Goodare, 1999; Kelson, 1999; Koops and Lindley, 2002; Telford et al., 2002). However, within the framework of biomedical research, it is the actual patients (in particular chronically or long-term ill patients), and not health care consumers in general, who may be relevant experts, since they have built up (bodily) experience with their diseases.

in the decision-making processes? Can these obstacles be overcome? In this dissertation I address these questions by examining the current role of patients in biomedical research decision-making, investigating any obstacles to the enhancement of this role and by searching for a possible strategy to overcome these obstacles. The presumption that patients, as stakeholders and potential experts, should participate in such decision-making is taken as a normative starting point.

In the next section of this chapter I describe the current situation of decision-making regarding biomedical research agendas, followed by an initial analysis of the role played by patients in this decision-making. The third section focuses on the concept of patient participation and elaborates on the different degrees of participation and the various objectives as reflected by the arguments for patient participation in biomedical research decision-making. The fourth section then describes the main research question and the research design of this study while the last section describes the outline of the book.

1.1 Biomedical research and its decision-making network

Biomedical research is a relatively new, fast-growing, and promising scientific research field that brings together fundamental and applied aspects of biology and medicine with the ultimate aim to contribute to the understanding and improvement of human (or animal) health, for example by searching for causes and working mechanisms of, or therapies for, pathological disorders. Sub-disciplines are, among others, pathology, immunology, human genetics, cell biology, microbiology, and neurosciences.[4]

Biomedical research can be considered as providing initial stages within overall biomedical innovation processes (Gelijns, 1991; Omta, 1995). Whereas basic biomedical research traditionally focuses on understanding physiological processes within the human body, applied biomedical research strives for the invention of new therapies, diagnostics,

[4] Contrary to definitions of biomedical research (implicitly) used by other scholars (e.g. Bondeson et al., 1982; Epstein, 2003; 2004; Nederbragt, 2000; Resnik, 2003) clinical trials are excluded from the definition used in this book. Clinical trials entail the testing rather than the invention and development of new technologies and thus can be considered a different stage within biomedical innovation processes (see also note 4).

etc. In contemporary biomedical science the majority of basic research has shifted towards 'strategic research', which combines scientific excellence with societal relevance, for example by searching for pathogenic mechanisms or causes of diseases (Rip, 2004). Further stages within the biomedical innovation process include technology development, clinical testing, production and diffusion (Gelijns, 1991; Nederbragt, 2000; Omta, 1995)[5].

The biomedical research field expanded considerably in the second half of the twentieth century. Developments within cell biology, molecular biology, and molecular genetics have strongly influenced medical thinking and acting[6], providing new insights in issues of health and illness and inspiring the development of innovative diagnostic and therapeutic tools. Moreover, diseases which are as of yet incurable, such as cystic fibrosis, Parkinson's, and cancer, might find treatment in the near future. For example, the Human Genome Project has caused an exponential growth in knowledge of the structure and function of human genes, which may lead to new possibilities for diagnosis, treatment and prevention of many diseases. The main barriers for the further implementation and diffusion of genetic technologies will be issues of societal adoption and regulation (Löhnberg et al., 1999).

In future, new and unforeseen problems may provide additional impulses for developments or changes within the biomedical research field. For example, the vast expansion of the Acquired Immune Deficiency Syndrome (AIDS) in the 80s, demanded new forms of national and international cooperation between researchers, physicians, and politicians, which has stimulated a more rapid diffusion of knowledge and methods and heightened discussions on national deficits in biomedical research. Obviously, these developments will also influence the way in which other diseases are investigated and handled (Löhnberg et al., 1999).

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[5] This description requires an additional comment. As has been generally accepted, relations between basic science, applied science, and technological development are complex and interactive rather than unidirectional (e.g. Dits and Berkhout, 1999; Kline and Rosenberg, 1986; Leydesdorff, 2000; Williams and Edge, 1996). However, since means and goals of, as well as possible stakes and roles of patients within, the different types of research differ, within the framework of this book for pragmatic reasons basic biomedical research, applied biomedical research, and clinical testing of biomedical technologies are (artificially) distinguished as different but interdependent stages of biomedical innovation processes (cf. Nordin, 1999; Omta, 1995).

[6] Within this framework one sometimes refers to the 'biomedicalization' of medicine (e.g. Clarke et al., 2003).

1.1.1 The biomedical research decision-making network

Within the framework of the study described in this book, it is important to find out who decides what research is to be conducted and what factors lay at the heart of these decisions. The various sub-disciplines of biomedical research are primarily conducted within laboratories and can be considered 'esoteric' disciplines that require the highly specialist expertise and skills of 'core-scientists'[7]. These scientists play a decisive role in determining research topics, questions, project planning and design, etc., and thus in the production of biomedical knowledge (e.g. Omta, 1995; Weissmann, 1982). However, scholars in the field of science and technology studies have shown that scientific knowledge (and thus biomedical knowledge) is not only the result of actions of, and interactions between, (core-) scientists. A variety of interactions with 'external' actors, such as the government, funding agencies, industry, etc., also strongly influence decision-making regarding science (e.g. Barnes, 1977; Bloor, 1976; Cozzens and Woodhouse, 1995). All these different stakeholders represent different social and political interests in biomedical research as well as different institutional contexts and cultures (cf. Elzinga and Jamison, 1995). Below, I describe the most important stakeholders involved in biomedical research decision-making processes in more detail.

The research community

As has been remarked above, a stakeholder group that plays a central role in decision-making on biomedical research topics, questions, design, etc., is the biomedical research community. This decision-making is strongly influenced by both internal and external factors (Schaffner, 1982). Internal scientific motives, such as scientific and technological feasibility and the adherence to scientific rules, determine individual research agendas (Weissmann, 1982). A range of external factors, such as personal interests and curiosities, scientific acknowledgement and prestige, political and financial support, societal needs and expectations, and social norms and values, play a very important role in decision-making on biomedical research agendas, as has been shown by several scholars in science studies, e.g. Marx Wartofsky (1982), Bruno Latour and Steve Woolgar (Latour,

[7] The term 'core-scientists' is borrowed from Harry Collins (1988; Collins and Evans, 2002) and refers to the group of scientists that is deeply involved in experimentation or theorization and thus contributes to the development of a certain scientific field.

1987; Latour and Woolgar, 1979), Karin Knorr Cetina (1981), and Joske Bunders (1994).

Besides the researchers themselves, their institutional contexts – biomedical research establishments such as universities, public and private research institutes, and industrial laboratories, as well as various inter-institutional and international research partnerships – play an important role in the steering of biomedical research. They provide a range of research facilities and decide on their own lines of research and research priorities. The influence of various institutional contexts on biomedical research varies per country. For example, while in the Netherlands a major part of biomedical research is conducted within academic laboratories, in the United Kingdom pharmacological firms and public research institutes play a much larger role (Cabello et al., 1999).

The government

A second stakeholder group consists of the national and international governments with their agencies, committees, councils, advisory bodies, etc. For these stakeholders, biomedical research is chiefly of interest for its potential social and economic uses. They decide on biomedical research legislation, administration, organization, and coordination.

In addition, federal funding agencies, such as the National Institutes of Health (NIH) in the United States, the Medical Research Council (MRC) in the United Kingdom, and the Netherlands Organisation for Health Research and Development (ZonMw) in the Netherlands, fund a large part of biomedical research. This governmental funding is especially important to guarantee that social health care needs are addressed and to stimulate the progress of biomedical science, since neither industry nor disease-specific charities fund basic biomedical science (Resnik, 2001). The funding agencies base the formulation of research priorities and programmes and the appraisal of individual research projects on both scientific (internal) and societal (external) criteria, such as the scientific quality of the researchers involved, the potential contribution to biomedical science, the burden of the disease addressed, possible institutional support, etc. (Blume and Catshoek, 2001; Cabello et al., 1999; Resnik, 2003; Van Hoëvell-van Dapperen, 1998). Officials involved in this decision-making are mainly biomedical experts, medical specialists, and government representatives. They are structurally advised by governmental councils or advisory committees and often seek additional advice from other sources, such as scientists, professional associations, patients'

organizations, industry, etc. (Resnik, 2001; Van Hoëvell-van Dapperen, 1998).

The industry

The pharmaceutical and biotechnological industry constitutes a third type of stakeholder in biomedical research and of growing importance. Companies spend large sums of money on biomedical research which aims to develop new drugs, medical devices, or biologics. Although many pharmaceutical companies still run their own biomedical research programme, they increasingly outsource strategic research to academic or private laboratories or research institutes (Meyer-Krahmer and Schmoch, 1998; Tijssen, 2004). The allocation of their money is mainly based on economic factors, such as market potential, liability costs, the scope of intellectual property protection, etc. (Hogg, 1999; Resnik, 2003).

The charities

Another group of stakeholders that is involved through funding consists of the private research funding agencies, such as the many disease-specific charities or foundations. They are created by patients' organizations, biomedical research communities, or medical professionals, to stimulate biomedical research on a specific disease or group of diseases. They usually raise their own (relatively small amount of) money to fund biomedical research projects. Main conditions for funding usually are the scientific quality of the project proposal and a correspondence with the formulated priorities and programmes (Blume and Catshoek, 2001; Cabello et al., 1999). Decision-making on funding is usually delegated to scientific advisory councils that consist of biomedical experts and medical specialists (Hogg, 1999; Van Hoëvell-van Dapperen, 1998).

The medical professionals

A stakeholder group that represents a different interest in biomedical research and influences decision-making in an indirect way is the community of medical professionals. Medical professionals can be considered as one of the user groups of biomedical research results. Over time biomedical developments have strongly influenced medical thinking. Within this framework, many medical professionals collaborate and communicate intensively with biomedical researchers, or may even themselves be researchers. Depending on the extent to which they

combine these roles they exercise varying degrees of influence on biomedical research agendas.

The patient community

Patients and their organizations form a group of stakeholders that is of particular interest in this dissertation. Although they are very involved in biomedical research as one of the end-user and target groups, they seem to be hardly involved in biomedical research decision-making processes. In the next section I will elaborate on the role of patients and patients' organizations in biomedical research decision-making in particular.

The public

Finally, the public at large can be considered a stakeholder of biomedical research, since it involves taxpayers who contribute indirectly to the governmental funding of basic academic biomedical research as well as constitute the potential supporters of charity funds. As the latter, members of the public can be said to influence decision-making on biomedical research agendas in a rather passive way. Their decision-making power is restricted to the choice of charity fund. Once they have donated their money, they leave it to the organization to spend it as it deems wise.

In addition, social pressure or interest groups, such as animal protection societies, may lobby or campaign effectively for either the stimulation or the suppression of certain types of biomedical research. Their influence is indirect, but may occasionally be substantial.

Over the last ten years or so biomedical research policy making increasingly involves the public in order to canalize social concerns and enlarge public trust in biomedical policy. Governmental organizations organize consensus conferences or public debates, such as the national debate concerning bioethics of embryo research in the United States (Kelly, 2003), the public consultation on developments in the biosciences in the United Kingdom (Irwin, 2001), and several consensus conferences on cloning (Van Est and Van Dijk, 2000).[8] In addition, some countries involve the general public in decision-making on biomedical research through special public advisory councils, such as the Consumer Liaison Group in the United Kingdom that aims to ensure the MRC is aware of,

[8] However, most consensus conferences on biomedical issues concern the diffusion and application of biomedical technologies, such as xenotransplantation, genetic therapies, etc. instead of biomedical research itself (e.g. Barns et al., 2000; CunninghamBurley et al., 2001; Dietrich and Schibeci, 2003; Einsiedel, 2002).

and able to respond to, consumer interests and concerns regarding research, and the Council of Public Representatives in the United States that advises the NIH on funding priorities; or via membership in decision-making councils, such as the NIH councils (Hagan, 2001; Resnik, 2001). Nevertheless, in spite of these forms of public involvement, in practice final decision-making on research funding is strictly determined by peer ratings of proposals (Cozzens and Woodhouse, 1995).

The network

The totality of actors and factors that determine biomedical research agendas can be considered a 'social network'[9], consisting of societal actors that are linked to each other by more or less stabilized relationships and interactions based on mutual exchange and dependency. Each actor plays its own role and has its own degree of involvement within the network (Elzen et al., 1996; Wellman, 1983). Kenneth Schaffner (1982: 141-142) describes this network as follows:

> *"What is decided to be desirable new knowledge in the biomedical sciences arises from a partially designed, partially serendipitous interplay among many of the groups and actors represented as nodes in the net, and involves conceivable alternatives, desired options, possible actions and outcomes, and judgements of feasibility and likelihood, as construed by many different parties".*

Within biomedical decision-making networks, the various funding systems have a very dominating influence on the steering of biomedical research (Cozzens and Woodhouse, 1995). However, the importance of

[9] In this dissertation, the concept of 'network' can be compared to the concepts of 'socio-technical network' and 'techno-economic network' as used by Elzen et al. (1996) and Callon et al. (1991) respectively. It should not be mixed up with the concept of 'actor-network' from the 'Actor-Network Theory', since only human actors and their mutual relations are considered. In fact, the biomedical research field consists of many different but strongly overlapping decision-making networks. In these networks finally emerging 'artefacts' are – instead of certain technologies as in socio-technical or techno-economic networks – individual biomedical research projects or programmes that are executed. Actors involved often take part in multiple networks, following general patterns of interaction. Since this study aims to analyze these general patterns within decision-making on biomedical research, it focuses on a kind of overall decision-making network that underlies all project or programme centred networks. Instead of individual persons, organizations, etc., the nodes of this network consist of clusters of actors (communities, types of organizations, etc.).

various funding sources differs per country. For example, in the United Kingdom, charities play a dominant role in funding biomedical research, whereas in the United States the NIH is by far the major source of funding for biomedical research. In the Netherlands, a large part of biomedical research is funded by general university funds and not bound to predetermined priorities or programmes (Cabello et al., 1999; Resnik, 2003; Van Hoëvell-van Dapperen, 1998).

As a result of differences in the structures of decision-making networks, the dynamics of steering varies per country as well. For example, while in the UK there is a lot of hierarchical steering by the charities and the MRC, in the Netherlands the influence of, and interaction between, the many intermediary organizations (advisory bodies and funding agencies) causes mutual adjustment rather than hierarchical steering (Cabello et al., 1999; Van Hoëvell-van Dapperen, 1998).

1.1.2 The role of patients in decision-making on biomedical research

Patients, although very involved in the biomedical research field as stakeholders, seem to be only marginally involved in biomedical research decision-making. Nevertheless, the fact that little has been published about the actual participation of patients in decision-making regarding biomedical research does not mean that patients and in particular patients' organizations do not occasionally strongly influence this decision-making. Some well-known examples concern the successful lobbying of patients' organizations for the stimulation of certain types of biomedical research at national policy levels (e.g. Kent, 2002; Parthasarathy, 2003; Rosengarten, 2004).[10] However, this patient activism does not entail the structural involvement of patients in formal research decision-making processes. Patients are only occasionally more structurally involved in research policy advisory structures, such as the Advisory Council on Health Research (RGO) in the Netherlands, which advises ZonMw on research funding

[10] Since patients are part of the public at large, they also may take part in public participation exercises within national biomedical research policy making. However public participation usually is inspired by political arguments mainly and does no right to the specific status of patients as stakeholders (beneficiaries) and experts. In addition it can be questioned how many patients are actually involved in those exercises. At most it can be considered a very limited and therefore not an adequate example of patient participation in decision-making on biomedical research.

programmes or priorities, and the European Platform for Patients' Organizations, Science and Industry that aims to influence European policy on health and health research matters.[11] Still, in those cases patients' actual influence on decision-making is hard to assess and may be questioned.

More direct examples of patients influencing biomedical research agendas concern patients' organizations that raise their own (small) research funds and formulate and implement their own biomedical research programmes (e.g. Blume and Catshoek, 2001; Kent, 2002; Rabeharisoa and Callon, 2002; Rangnekar and Duckenfield, 2002). A rather well-studied example is the case of the German Retinitis Pigmentosa (RP) patients' society Pro Retina, which was quite successful in stimulating and influencing biomedical research practices in Germany. It had formulated and implemented its own research priorities, intervened in the scientific community, lobbied for public funding, and initiated innovative research projects (see box 1.1). However, since the implementation of the initial RP research program, decision-making about research funding has been delegated to the scientific advisory board, which consists of scientists only. This board bases its decisions on standard criteria of biomedical research and usually does not allocate funds to non-conventional ideas or projects.

Box 1.1 The case of Pro Retina*

Pro Retina is the German patients' society for Retinitis Pigmentosa (RP), which can be described as any of several hereditary degenerative diseases of the eye that usually leads to total blindness. The main aims of Pro Retina are to support research on RP with respect to diagnosis and therapy, exchange information and experiences among RP patients, inform the public about RP and its social consequences, and exert influence on public and private persons, organizations, and institutions.

Soon after its establishment in 1977, Pro Retina discovered that in Germany no systematic biomedical research was being conducted on the cause of the disease and possible therapies. Therefore, it decided to encourage the scientific community and to stimulate systematic research on RP. For this purpose Pro Retina published a report on the international state of affairs in RP research, installed a research referee that was to form a bridge between patient and research communities, established a RP research foundation trust at the German Science Foundation, and established a scientific advisory board (mainly

[11] see http://www.rgo.nl and http://www.epposi.org

concerned with the prioritization and commissioning of research projects on RP).

In addition, Pro Retina organizes national and international conferences on RP. The first conference organized by Pro Retina took place in 1984. During this conference, characterized by many interactions both between researchers from different disciplines and between researchers and patients, Pro Retina played a central role in the prioritization of RP research. Patient priorities that were expressed included research on the biochemical and genetic causes of retinal degeneration, research on the efficacy of current and future treatments of RP, the improvement of everyday visual conditions for RP patients, and the improvement of conditions for effective genetic counselling. Based on these priorities, the scientific advisory board established an integrated RP research programme.

The growing implementation of RP research programmes has been possible because of Pro Retina's successful lobby for public funding. As a result in Germany there now is a flourishing RP research community with international prestige. Apart from prioritizing and funding research, Pro Retina is also interested in directing actual research projects to meet the needs and requirements of RP patients, for example by inviting specific researchers to congresses and seminars, or by financing part of their work. One of the recent research priorities of Pro Retina is molecular genetics.

Pro Retina adopts a flexible attitude toward new and unconventional ideas that might not be accepted by the established research community but might give rise to promising innovations in the fight against RP. In order to give these ideas a chance, Pro Retina occasionally provides funding without consulting its scientific advisory board first.

* A more detailed description of this case has been published before by Flinterman et al. (2001). It is based on Von Gizycki (1987), Bunders (1994), Pro Retina (1999), and personal communication with H. Gusseck, research contact person at Pro Retina, on February 7, 2001.

Similar to Pro Retina, most patients' organizations that financially support biomedical research have established scientific advisory committees in order to deal with the formulation of research programmes and the appraisal of research projects (e.g. Blume and Catshoek, 2001; Flinterman et al., 2001; Hogg, 1999; Rabeharisoa and Callon, 2004; Rangnekar and Duckenfield, 2002). In addition, many patients' organizations have professionals on their administration board. As a consequence the involvement of patients' organizations in funding biomedical research does not necessarily imply the actual involvement and influence of patients (as both stakeholders and experts) on biomedical research agendas.

In the literature, only very few cases have been described concerning patients' organizations that actually involve patients in decision-making on research programmes or priorities in a structural way. Maybe the two most well-known examples are the Alzheimer's Society in the UK (Alzheimer's Society, 2002; 2004; Kent, 2002) and the French Muscular Dystrophy Association, which has been extensively studied and described by Rabeharisoa and Callon (2002; 2004). Within the Alzheimer's Society in the UK, the programme 'Quality Research on Dementia' is an active partnership between carers, people with dementia, and the research community. The heart of Quality Research in Dementia is the QRD Advisory network: a network of 150 carers, former carers, and people with dementia who play a full role in determining the strategy for research, providing comments and prioritization of grant applications, selecting applications for funding, monitoring on-going projects being funded by the Society, and dissemination of research results.[12] Each year they decide on research priorities for the programme, which have to be actively pursued by research applicants (Alzheimer's Society, 2002).

The French Muscular Dystrophy Association (AFM) is a patients' organization focussing on neuromuscular diseases. Since its creation in 1958 it has become one of the main funding institutions of molecular-biological research in France. Diseases that were once orphan, now are surrounded by influential research communities. But unlike most other patients' organizations, the AFM did not delegate research decision-making to scientists once the diseases were recognized and investigated. From the outset, patients and their families have maintained full control over both the association and the orientation of research. The AFM combines the mobilization of research communities around neuromuscular diseases with the active participation of patients and their families in the orientation of biological and clinical research and the production of knowledge on these diseases (see box 1.2).

At the level of individual projects, patients' roles are usually restricted to supplying research material and being the ultimate target group of research outcomes. Examples of patients that influence individual research topics or questions often remain anecdotic (e.g. Chalmers, 1995).

[12] see http://www.qrd.alzheimers.org.uk/consumers.htm (accessed at December, 4, 2004)

Box 1.2 The case of the French Muscular Dystrophy Association[*]

The French Muscular Dystrophy Association (AFM – "Association Française contre les Myopathies") is a grassroots organization for patients with neuromuscular diseases and their parents, which was founded in 1958 as the successor of a number of small-scale support groups. Since at that time only little knowledge on the various and rare diseases was available, members of the AFM started to produce knowledge themselves by making films, photographs, and testimonies about the characteristics and progression of the diseases.

Today, the AFM has become one of the main funding institutions of molecular-biological research in France. One of the most important impulses of its growth was the launching of the 'Telethon' in 1987, a very successful annual TV-fundraising campaign that raises large amounts of money used to support and influence research. In its support of research the AFM has devoted itself principally to biomedical (including genetic and 'post gene') research.

The AFM has itself defined research as a process that should start with the needs of the patients, and eventually return to them by proposing a solution. In order to ensure that research meets the demands and expectations of patients, the AFM administrators acknowledged the need for intensive interaction between researchers and patients. Therefore the AFM has increased the number of meetings, discussions, and forms of cooperation between patients, scientists, and clinicians. At the same time the association has conceived various political and organizational devices to ensure that, in all this interaction and cooperation, roles and responsibilities are not confused; the power of decision must remain in the patients' hands.

Besides an interdisciplinary scientific council that advises on research funding, the AFM has established a strategic council of a small number of experts with diverse competences, who can be consulted on all kind of issues. In regular contact with these experts the association forms an opinion on the most promising routes towards treatment, which serves as a basis for strategic decisions. In spite of the pressure of certain members of the scientific council to bring the research more in line with their own professional interests, the AFM board has consistently refused to relinquish any of its strategic power.

An example of this strategic power was the launching of the Genethon laboratory in 1991 (comprising a large research project that aimed to locate and identify the genes responsible for neuromuscular diseases) and soon after, the AFM's engagement in therapeutic research. Although the launching of the Genethon was not acclaimed by the majority of scientists within the scientific council, it has gained an international reputation after providing the first physical maps of the human genome in 1993.

[*] The description of this case is based on Rabeharisoa & Callon (2002; 2004) and Blume & Catshoek (2001).

1.2 The concept of patient participation

Thus far, the concept of *patient participation* has not yet been problematized. However, in the framework of this study it is essential to define patient participation further and to elaborate arguments for patient participation in decision-making on biomedical research.

As stated above, patients are *stakeholders* and potentially relevant (non-certified) *experts* within the framework of biomedical research. Therefore patient participation in decision-making on biomedical research can be considered a form of stakeholder participation. Many definitions have been formulated for stakeholder participation, differing in the interpretation of the terms 'stakeholder' (individual or group; having a clear stake or being affected; etc.) and 'participation' (passive sharing of information or active power sharing; direct or delegated; etc.). In this dissertation, I use a broad definition of stakeholder participation, namely 'any initiative that aims to involve stakeholders in decision-making on a plan, policy, or problem-solving process beyond that of voting in elections' (adjusted from Smith, 1984). Thereby a stakeholder is anyone (individual or group) who has a stake or interest in the issue or decision under discussion.

In this study, patient participation in biomedical research decision-making is therefore defined as any initiative that aims to involve patients in that decision-making process. Patient participation could in principle occur at several levels of decision-making on, and at several stages within, biomedical research processes. For example, at a policy making level, patients might participate in the making of decisions concerning themes, rules, programmes, priorities, strategies, etc., while at the level of individual research projects, patients might be involved in decisions on specific research topics or questions. Although patients might not be in the position to participate directly in decision-making at the stages of design, execution, or result analysis of individual biomedical research projects, they could probably contribute to decisions concerning *biomedical research agendas*, whether these are individual, institutional, or national.

Patient participation initiatives could be described and mutually distinguished along two dimensions. The first dimension concerns the *degree* of participation (degree of influence or power sharing) that is to be achieved. Stakeholder participation methods described in literature include forms of communication or information, stakeholder consultation, stakeholder membership in advisory or decision-making structures,

deliberative approaches, etc., all reflecting different degrees of stakeholder input and influence (e.g. Oliver et al., 2004; Rowe and Frewer, 2000; Van Asselt and Rijkens-Klomp, 2002).

The second dimension concerns the *objectives* which are reflected by the underlying rationales for participation and that implicitly or explicitly inspire and shape participation initiatives. Objectives of participation can vary from the enhancement of the legitimacy of decision-making processes, to the realization of more sophisticated outcomes or the achievement of a higher societal acceptation of the outcome. In the following subsections these two dimensions of patient participation in biomedical research will be studied in more detail.

1.2.1 Degrees of participation

The term *'participation'* can be related to terms like 'involvement' and 'communication'. Participation and communication both can be considered two forms of the broader concept of involvement[13]. The term 'communication' refers to a more passive involvement and is often associated with the so-called 'deficit model' (Durant et al., 1992; Ziman, 1991). This model assumes that the stakeholder involved lacks sufficient knowledge or capacities to be able to participate adequately in decision-making processes. Many stakeholder communication methods aim to develop stakeholders' understanding of issues concerned, and to align their visions with the visions of experts, in order to gain a higher acceptance of policies or decisions (Rowe and Frewer, 2000). By contrast the term 'participation' is generally considered to refer to a more active involvement in decision-making processes. Participation methods often start from the assumption that the stakeholder involved has useful knowledge or perspectives that can contribute to policy or decision-making (e.g. Fiorino, 1990; Nordin, 2000; Van Asselt and Rijkens-Klomp, 2002; Wright, 1976).

In order to utilize the knowledge from stakeholders in an optimal way, stakeholders need to be directly involved in decision-making processes. Also Collins and Evans (2002: 262) argue that expert-stakeholder participation in decision-making requires a direct, non-delegated involvement; not by survey but by action. That applies in particular to experience-based experts, whose contributory expertise is

[13] In the literature the term 'patient participation' is often used synonymously with terms like 'patient involvement' or 'consumer involvement' (see also note 1). I however consider participation as one of the many forms of involvement.

partly implicit. In addition, the decision-making process should ensure that stakeholders' knowledge is actually integrated in the outcome. At the same time (genuine or effective) participation is often associated with values such as fairness, competence, equality, mutual respect, and mutual learning (e.g. Abelson et al., 2003; Jacobson and Storey, 2004; Webler and Renn, 1995; Webler and Tuler, 2000). In doing so reference is usually made to Jürgen Habermas's 'theory of communicative action' that involves action based on consensus among all interested and affected parties in collaborative decision-making processes, achieved by free and rational discourse (Habermas, 1975; 1984; 1987).

However, evaluation studies have shown that not all participation methodologies bring about direct, influential, and fair public or stakeholder participation to the same extent (e.g. Abelson et al., 2003; Fiorino, 1990; Guston, 1999; Pratchett, 1999; Rowe and Frewer, 2000). Different methodologies reflect different *degrees* of participation, comprising different degrees of influence or power sharing.

In order to be able to distinguish different degrees of patient participation, Sherry Arnstein's 'Ladder of citizen participation' is used as a framework (1969). Arnstein defines participation as "the redistribution of power that enables the have-not citizens, presently excluded from the political and economic processes, to be deliberately included in the future" (p. 351). Her 'ladder' consists of eight rungs that stand for eight levels of citizen participation in local policy making (see figure 1.1). These levels reflect different degrees of power distribution, ranging from 'manipulation' to 'citizen control'. The three lowest rungs of the ladder (*manipulation, therapy,* and *information*) reflect levels of communication[14] rather than participation, leaving all decision-making power to traditional policy makers and denying citizens any influence on the outcome. Therefore these levels are left out of the further analysis. The five highest degrees of participation all reflect some input of citizens, albeit with different influence on decision-making. In table 1.1 these five degrees are translated into terms of patient participation in biomedical research.

[14] Arnstein regards 'information' as a form of tokenism rather than as non-participation. However, since information comprises a one-way communication, usually taking place late in decision-making processes, it hardly provides any room for stakeholders to influence the decision-making. Therefore I have left out this level of participation from further analysis as well.

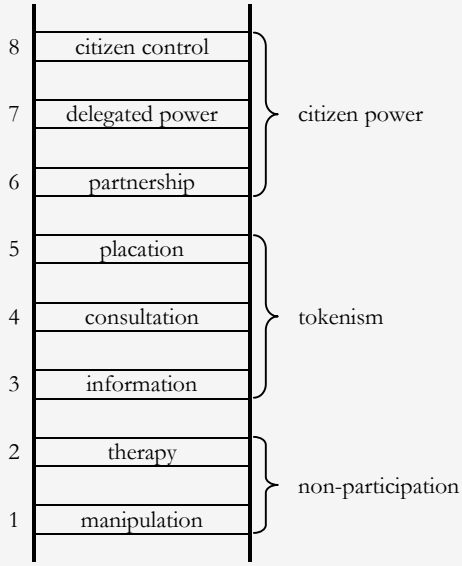

Figure 1.1 Arnstein's Ladder of Citizen Participation (1969)

The two levels of *consultation* and *placation* reflect low degrees of participation and are often conducted with the aim of legitimization or satisfaction ('tokenism'). Consultation takes place when patients are consulted for their perspectives by means of a survey or (group) interview. Placation concerns the partaking of patients in formal advisory or decision-making committees or boards. In both cases patients are allowed to express their views and feel that they are being heard but any actual influence on outcomes is not guaranteed. Professionals (scientific experts or policy makers) ultimately decide if patients' inputs are included in actual decision-making. Therefore, these levels cannot be considered genuine or effective participation.

Following Arnstein, *partnership* consequently can be considered the minimal level of genuine patient participation, reflecting actual power sharing in fair decision-making processes that incorporate negotiation and deliberation. It implies equality and mutual respect between participants. This degree of participation is the one implicitly or explicitly addressed by many scholars concerned with (effective) public participation and (deliberative) democracy. It can be considered as reflecting the earlier

mentioned theory of communicative action by Jürgen Habermas (1975; 1984; 1987).

Table 1.1 Five levels of patient participation in decision-making o ı biomedical research, as based on Arnstein (1969).

I *tient control*	Patients control decision-making on biomedical research age das.
I *legated power*	Patients gain a dominant position in the decision-making p: ›cess to ensure the accountability of research programmes or projects to the needs.
I *rtnership*	There is actual power sharing between patients and prof ssionals within decision-making processes, for example in joint polic or planning committees. Final outcomes are the result of genuine nego: ations between both parties.
I *ucation*	Patients participate in decision-making or advisory structu es in a formal way, without any guarantee that their inputs are being hono: ed. In practice professionals dominate the actual decision-making.
C *nsultation*	Patients are consulted for their research needs, judgements priorities, etc., without implying that these inputs are actually taken int consideration. Professionals decide on whether or not to use patients' nputs in their decisions.

A still higher level of patient participation is *delegated power*, which refers to a situation in which patients rule certain parts in the decision-making process. They for example get appointed decision-making authority on certain issues or a kind of veto-power. The delegated research priority setting within the QRD programme of the Alzheimer's Society in the UK could be considered a form of delegated power.

The highest level refers to patients' *control* of decision-making on biomedical research. Arnstein admits that absolute citizen control of decision-making is not realistic, nor is absolute control of decision-making on biomedical research by patients. Absolute patient control over biomedical research may endanger the professional autonomy of biomedical researchers and might result in the loss of scientifically important basic research directions. In addition, it might not be labelled participation since, according to Wright (1976), in genuine participation "no single person [or community] tends (by definition) to control the decision-making process to any extent" (p. 232). Participation requires at least some interchange among participants. Therefore, Arnstein's highest level of participation refers to the control over certain programmes or institutions instead of absolute control.

The different levels of patient participation exhibit some overlap. For example, the partaking of patients in decision-making structures can comprise either placation or partnership, depending on the actual power balance between professional and patient participants. In practice, however, it is usually very difficult or even impossible to distinguish placation from true partnership, since the actual influence of patients in decision-making processes within these structures is hard to estimate (see Oliver et al., 2004: 88, 90-91). Within the broader field of patient participation, one therefore often uses the term 'collaboration' to refer to patients' active involvement in decision-making structures, without pronouncing on patients' actual influence on decision-making (e.g. Oliver et al., 2004; Paterson, 2003; Telford et al., 2002).

In addition, participation exercises may show characteristics of more than one level. For example, certain elements within the case of the French Muscular Dystrophy Association (box 1.2) could be considered as reflecting partnership while other elements indicate a form of patient control.

Other scholars have proposed other frameworks that differentiate levels or degrees of participation (e.g. Biggs, 1989; Pretty, 1995). In the context of patient participation in biomedical research an interesting framework is presented by Michel Callon, who describes three models of lay participation in scientific and technological decision-making (1999). Instead of focusing on power distribution like Arnstein, Callon focuses on knowledge distribution within decision-making. His first and lowest level is the 'Public Education Model' that refers to the information and education of the scientifically illiterate public, thus enhancing the lay people's trust in science. The second level, the 'Public Debate Model', refers to the participation of lay people in scientific debates and is based on the idea that the local or contextual knowledge of lay people could enhance and complete scientific knowledge. However, the demarcation between science and non-science remains intact. Callon's third level, the 'Co-production of Knowledge Model', finally, refers to the active involvement of lay people in the creation of knowledge that concerns them. Knowledge production takes place in close collaboration between scientists and lay people, each having maintained their own tasks, but with the group of lay people as an 'obligatory point of passage'. It is characterized by collective learning on an equal basis (Callon, 1999). Although there are some similarities between the frameworks of Arnstein and Callon, in this study I use Arnstein's participation ladder since it offers a somewhat more extensive differentiation of levels of participation.

1.2.2 Objectives of participation

As stated above, stakeholder participation initiatives are undertaken with different implicit or explicit aims constituting the rationales for participation. Participation can be considered the means to an end or an end in itself and can be morally, politically, instrumentally, or substantively motivated (Abelson et al., 2003; Fiorino, 1990; Flinterman et al., 2001; Telford et al., 2002; Webler and Renn, 1995). Normative (moral or political) arguments for stakeholder participation in decision-making, used by different scholars in the field of participation studies, usually reflect objectives such as the enhancement of the legitimacy of the decision-making process itself or the accomplishment of some power rebalancing (stakeholder empowerment). Substantive arguments focus on the actual contribution stakeholders can make to the decision-making process and thus to the rationality of the process and the quality of its direct outcome. More instrumental arguments usually refer to the achievement of social acceptance (and the avoidance of conflicts) of both the process and its outcomes, or to the accomplishment of societal knowledge sharing (e.g. Abelson et al., 2003; Bickerstaff and Walker, 2001; Collins and Evans, 2002; Irvin and Stansbury, 2004; Jasanoff, 2003; Renn et al., 1993; Webler and Renn, 1995; Webler and Tuler, 2002). These instrumental arguments are used by governmental or research-related organizations that have a particular interest in stakeholder participation.

All arguments thus reflect certain objectives that can inspire stakeholder participation initiatives. These objectives concern either the decision-making process itself or its (direct or indirect) outcomes. The objectives that focus on the process and its direct outcomes can be considered short-term objectives that concern the particular context of the participation initiative. The objectives that refer to societal knowledge sharing or power rebalancing are long-term aims that may eventually transform societal networks. The different objectives are now further elaborated within the framework of patient participation in decision-making on biomedical research agendas.

1. Enhancement of decision-making legitimacy

One of the most frequently mentioned objectives of patient participation in the decision process is that it enhances the legitimacy of the process itself. A decision-making process can be considered legitimate if its justness is generally acknowledged. This justness can concern the people involved in decision-making as well as the procedures followed. The

acknowledgement of justness can be based on different grounds. Legal legitimacy, for example, concerns acknowledgement on the basis of consistency with current legislation, while normative legitimacy refers to the acknowledgement of justness of a process on normative grounds. Although patient participation might not easily contribute to legal legitimacy of the biomedical research decision process (except when a plea for democratization of biomedical research is somehow incorporated in legislation), it certainly might contribute to its normative legitimacy on the basis of different normative claims.

An often mentioned normative claim, pleading for stakeholder participation in the decision process in general, is that those affected by a decision should have input to that decision (e.g. Fiorino, 1990; Smith, 1984). This claim implicitly refers to basic values of human self-determination and autonomy and the assumption that stakeholders (citizens, end-users, etc.) are the best judge of their own interests, which goes back to John Stuart Mill's ideas of social liberty (Mill, 1869). Because patients' lives can be strongly affected by biomedical research results and outcomes (e.g. by means of new therapies or medicines), patients could be ascribed the right to have a say in biomedical research and development processes (Barbour, 1992; Bastian, 1994; Goodare and Smith, 1995).

Another normative value pleading for patient participation in the decision process on biomedical research is the ideal of direct democracy, dating back to Rousseau (1762). Democracy can be considered an essential value within our political culture that calls for the democratization of public goods and thus of science (Feenberg, 1995; Fiorino, 1990; Fuller, 2000; Jasanoff, 2003; Webler and Renn, 1995). Since a large part of biomedical science is publicly funded and can be regarded as a public good[15], decisions on its direction should involve a democratic process with all relevant stakeholders or even the public at large.

2. Enhancement of decision-making rationality

A possible objective that addresses another quality of the decision-making process is the enhancement of the rationality of biomedical research decision-making. The sociologist Max Weber (1968) distinguishes two forms of rationality of action that apply in this context: goal-oriented (or

[15] Research funded by charity funds, although not a public good in the strict sense, has a kind of constituency consisting of members and supporters that provide the money. This constituency can be ascribed a voting right, thus pleading for a degree of democratization as well.

instrumental) rationality and value-oriented rationality, which both refer to the intention of action, respectively a strategic or utilitarian intention and a value-based intention (Oakes, 2003; Weber, 1968). The goal-oriented rationality of a decision-making process refers to its purposefulness, effectiveness, and efficiency and thus indirectly to the adequacy of alternatives and criteria considered and to the ultimate costs and benefits. The value-oriented rationality refers to the consistency of decision-making procedures with social value systems. The objective of rational decision-making thus can form the basis of both substantive and normative arguments.

Patient participation could contribute to some aspects of goal-oriented rationality in biomedical research decision-making. For example, patients could increase the purposefulness of decision-making processes by helping to specify the purpose a research programme should adhere to and ensuring that purpose addresses societal needs (cf. Resnik, 2001). They can also contribute to the effectiveness of decision-making by introducing relevant alternatives and weighing criteria that professionals may overlook, and by steering clear of potential negative consequences that professionals may not be aware of (cf. Jasanoff, 2003). In addition, patient participation can contribute to the value-oriented rationality of decision-making on biomedical research, because it ensures consistency with social values such as direct democracy and patients' rights to participate (cf. Renn et al., 1993; Thomas, 1984). However, patients' contribution to the efficiency of decision-making is less obvious. In this respect, biomedical research decision-making differs from other decision-making contexts, which require stakeholders' agreement for successful implementation of decisions, e.g. environmental planning, sustainability development, or clinical research. Within these contexts, early stakeholder participation is necessary to avoid dragging conflicts, stakeholder resistance, or objection procedures afterwards, thus avoiding extra costs and enhancing the efficiency of decision-making processes (Irvin and Stansbury, 2004).

3. Increase of outcome quality

A third objective of patient participation in biomedical research decision-making is to increase the quality of the outcome of the decision-making process. This objective constitutes a substantive argument that pleads for patient participation because it may lead to 'better' decisions, in terms of more socially relevant, more inclusive, or more appropriate to the context of application. This argument refers to the experience-based knowledge

that patients[16] can contribute to the decision-making process and that complements professional biomedical knowledge by providing alternative views on matters of health and illness (Entwistle et al., 1998; Goodare and Smith, 1995; Popay and Williams, 1996; Telford et al., 2002). This knowledge can be considered 'contributory expertise' in terms of Collins and Evans (2002). It could include alternative perspectives on the societal relevance of certain research priorities or topics. An Australian study, for example, shows that breast cancer survivors made the topics "risk factors" and "diagnosis" their highest priorities for research whereas clinicians and researchers were more focused on the genetic basis of the disease and on the development and evaluation of new treatments (Marlin et al., 1996). Other scholars also have found that patients' research priorities differ from the priorities of professionals (Grant-Pearce et al., 1998; Griffiths et al., 2002; Tallon et al., 2000; Van der Wilt, 1995). In addition, patients' experience-based knowledge could offer alternative and useful insights or ideas on etiological questions, triggers for symptoms, the impacts of symptoms on daily life, patients' needs for and values concerning technologies, etc. (Entwistle et al., 1998; Popay and Williams, 1996; Van der Wilt, 1995). By sharing these ideas, patients can help researchers to identify new research questions and to formulate hypotheses.

Integration of patients' experience-based knowledge broadens decision-making on biomedical research and that may eventually give rise to the adoption of alternative, more relevant research directions or the development of more appropriate technologies (Van Kammen, 2000). As has been argued by Van der Wilt (1995), the omission of patients' input in decision-making on biomedical research agendas easily results in an one-sidedness of research programmes, that does not take into account professional uncertainties, ambiguities, and pluralisms, or meet patients' most urgent needs.

4. Increase of social acceptance

A possible instrumental objective of patient participation in decision-making on biomedical research is related to the general rationale applied to public participation in decision-making that informing and involving the public will enlarge its confidence in and support of final outcomes, thus avoiding societal conflicts (Beierle and Konisky, 2000; Bickerstaff and Walker, 2001; Feenberg, 1999; Irvin and Stansbury, 2004; Mitcham, 1999;

[16] in particular the (semi-)chronically ill patients who have repeatedly or long-lasting experienced their diseases

Rowe and Frewer, 2000; 2004). These outcomes might concern (biomedical) research agendas but also resulting technologies. Since the second half of the twentieth century, problems around the societal diffusion and application of certain new biomedical technologies have shown that societal acceptance of biomedical technologies is not always a self-evident matter (Barns et al., 2000; Mendelsohn et al., 1979; Van Est and Van Dijk, 2000). Involving stakeholders in decision-making at an early stage of research and development processes might avoid such societal conflicts (e.g. Koch, 1995).

The social acceptance of a decision is correlated to the social perception of a fair decision-making process (Renn et al., 1993) – also referred to as the social legitimacy of the decision-making process. This in turn can be considered as dependent on both the (legal and normative) legitimacy and the rationality of the decision-making process. However, since the social acceptance of decisions often has been used as a primary and instrumental argument for stakeholder participation, here it is mentioned as a separate objective of patient participation in biomedical research decision-making.

5. Increase in human capital

Besides the short-term objectives described above, a long-term objective of patient participation in biomedical research decision-making could be the sharing of knowledge between patients and professionals, which might result in an increase in societal human capital. Human capital can be defined as "the knowledge, skills, competences and other attributes embodied in individuals, which facilitate the creation of personal, social and economic well-being" (Côté, 2001: 30).[17]

Effective participation of patients in decision-making processes on biomedical research results in an integration of patients' experience-based knowledge and perspectives with scientific and professional knowledge and in mutual learning between both parties involved (Broerse and Bunders, 1999; Flinterman et al., 2001). Whereas patients may learn about

[17] Although the concept of human capital usually refers to the economic values of individuals in particular, which can be assessed by measuring duration of schooling and qualification levels (OECD, 1998; Schuller, 2001, The Penguin Dictionary of Economics, 1984), within our framework we use a somewhat broader definition that includes motivation, moral behaviour, and attitudes, and thus tacit and inter-personal knowledge (Côté, 2001).

scientific (biomedical) knowledge, perspectives, and procedures, etc., professionals might learn from patients' perspectives, experiences, and needs. The second direction of learning is comparable with what Daniels and Walker have described as 'social learning' (1996). Both parties thus benefit from participation processes, resulting in an overall increase in human capital that enables a more adequate societal response to contemporary complex questions (Irvin and Stansbury, 2004). Or, as Sheila Jasanoff (2003: 398) states: "participation can serve to disseminate closely held expertise more broadly, producing enhanced civic capacity and deeper, more reflective responses to modernity". Moreover, the resulting reduction in language and knowledge gaps between patient communities and research communities can smooth future participation processes.

Another increase in human capital concerns the general increase in knowledge on patient participation processes. Successful patient participation initiatives may result in the learning of both participants and other actors indirectly involved about methods and possible benefits of patient participation in biomedical research decision-making. This increase in knowledge may facilitate future participation processes as well.

6. Increase in social capital

Another possible long-term objective of patient participation in decision-making on biomedical research is that it might bring about patient empowerment (Boote et al., 2002; Morrissey, 2000; Webler and Renn, 1995) and decrease the power imbalance between patients and biomedical professionals. Eventually this 'power sharing' might result in a more equal, cooperative, and beneficial relationship between the patient community and the biomedical research community, characterized by mutual respect, trust, and understanding, as well as in an expansion of biomedical research decision-making social networks. In this way patient participation could contribute to a general increase in social capital (Abelson et al., 2003; Coleman, 1988a; Veenstra and Lomas, 1999).

The objective of patient empowerment forms the basis of normative, socialistic arguments. However, since the social capital of a society is generally regarded as an important factor that enhances economic success and social well-being (Côté, 2001; Putnam, 2001; Veenstra and Lomas, 1999), this objective can inspire more pragmatic arguments as well.

In table 1.2 the different objectives for patient participation in decision-making on biomedical research are summarized.

Table 1.2 Objectives of patient participation in decision-making on biomedical research

Focus	*Objective*	*Main related arguments*
proces s	decision-making legitimacy	normative (moral and political)
	decision-making rationality	normative and substantive
short-term outcome	outcome quality	substantive
	social acceptance	instrumental
long-term outcome	human capital	instrumental
	social capital	normative and instrumental

Relation between objectives

The six objectives mentioned above are closely intertwined. The first two objectives can to a certain extent be viewed as preconditions for the latter four. For example, the quality of a decision strongly depends on the rationality of the decision-making process, while its social acceptance depends on both the legitimacy and rationality of the process. Furthermore, the more rational a decision-making process is (in terms of including all knowledge and perspectives available) the higher its possible contribution to human capital, while both decision-making legitimacy and rationality may contribute to patients' empowerment, and – thus – to social capital.

In addition, increases in social capital and human capital are narrowly related. On the one hand patient empowerment (which can be related to an increase in social capital) is often thought to depend on the increase of patients' knowledge of their diseases (cf. D'Alessandro and Dosa, 2001; Johnston Roberts, 1999; Weiner, 2003). On the other hand increases in social capital can facilitate processes of societal knowledge exchange (Coleman, 1988b), thus contributing to an increase in human capital.

1.2.3 Arguments contra patient participation

All objectives described above encompass arguments in favour of patient participation in biomedical research decision-making. However, there are also some arguments against patient participation. One often-heard argument is that participation exercises are expensive and time consuming

and thus strongly decrease the cost-effectiveness of decision-making processes. Other counter arguments are that lay participants are too irrational, ignorant or incompetent to make reasonable decisions on technical issues (Futrell, 2003).

Another argument against patient participation in biomedical research in particular is that biomedical research is an 'esoteric' science that should not be interfered with, especially not by lay people who lack the specialist knowledge necessary to contribute anything relevant to biomedical knowledge production. Supporters of this opinion usually refer to important medical breakthroughs that have been the result of basic biomedical science inspired by scientific curiosity only, such as the discovery of bacteria and the discovery of DNA and its structure (e.g. Weissmann, 1982). Indeed, the intrinsic and potentially utilitarian value of isolated, basic biomedical research should not be underestimated. For this reason the question could be posed if all basic biomedical research needs to be submitted to the influence of patients. Indeed, several scholars who study the democratization of science and technology stress that more participation is not always better (e.g. Collins and Evans, 2002; Resnik, 2001; Rip, 2003). For each situation an optimal balance needs to be found between excluding patients from any influence and letting them exercise total power. This optimal balance could very well turn out to be different for basic and applied biomedical research.

1.3 Research design

A first literature search on the topic, resulting in the overview in section 1.1.2, strengthens the aforementioned assumption that patient participation in biomedical research decision-making is exceptional and marginal rather than structural and widespread. Patients seem to be excluded from the biomedical research decision-making network. If this assumption is correct, the question arises why the practice of patient participation in the biomedical research agenda setting is lagging behind other forms of stakeholder participation in decision-making regarding science. Do patients themselves, biomedical researchers, or other actors involved ward off (consciously or unconsciously) active patient participation in decision-making on biomedical research? If so, what are their motives and are these motives valid? If not, what other obstacles hamper patient participation in biomedical research agenda setting? These

questions ask for/require more research on the current state of patient participation in biomedical research decision-making.

Subsequently, the different arguments that plead for active patient participation in decision-making on biomedical research agendas justify additional research that addresses conditions and strategies that can remove or overcome these obstacles and contribute to effective patient participation in decision-making on biomedical research.

Within this framework I have formulated the following **main research question** that is addressed in this book:

> *"To what extent is effective patient participation in biomedical research decision making possible?"*

In order to answer this question, I have formulated **three sub-questions** that determine the outline of the study:

I. What causes the apparent limited participation of patients in decision-making on biomedical research agendas?

II. What strategy could be devised to include them more actively and effectively in biomedical research decision-making processes?

III. What can we learn from the practical implementation of this strategy, in particular in terms of effectiveness?

Since these sub-questions are qualitative questions, answering them demands a qualitative research design. This study followed a so-called *interactive model* of research design (Maxwell, 1996; 1998). Central to this model is the idea that the essential elements of a study (context, objectives, research questions, methods, and validity checks) form an integrated whole, rather than being linked to each other in a linear or cyclic sequence. The research questions form the centre of the model. On the one hand they are inspired by the objectives of the study and informed by the cumulating knowledge about the context in terms of theories and findings. On the other hand they take into account the feasibility of methods and the seriousness of particular validity threats.

The broader societal and conceptual context of this study on patient participation in biomedical research decision-making has already been described in the previous sections. Knowledge and theories on additional contextual aspects, such as current strategies to realize patient participation in biomedical research decision-making, the potential value of patients' experience-based knowledge for this decision-making, factors that hamper or facilitate effective patient participation in this decision-making,

etc., will be developed during the study. Below I will describe the objectives of the study, its scientific and societal relevance, the research questions, the methods used, and the validity checks which were built in.

1.3.1 Objectives and relevance of the study

The primary *objective* of the study is to reflect on and experiment with patient participation strategies, building on the experiences of both current patient participation initiatives and successful stakeholder participation strategies that are applied within other scientific fields, in order to gain increased insight into how to design an appropriate strategy[18] for effective patient participation in biomedical research decision-making. Sub-objectives within this framework are increased understanding of (1) the validity and value of patients' experience-based knowledge and its relation to scientific biomedical knowledge; (2) obstacles that hamper effective patient participation in biomedical research decision-making; and (3) conditions and methods conducive to the implementation of effective patient consultation and participation within the biomedical field.

The study described in this dissertation is both scientifically and societally relevant. The *scientific relevance* is that it contributes to the on-going debate on one of the central themes within the field of Science and Technology Studies (STS): broadening decision-making processes concerning science and technology. The *societal relevance* of the study is that it contributes to the formulation and practical implementation of a strategy for effective patient participation in decision-making on biomedical research, which may eventually contribute to the induction of a transition of the biomedical research decision-making network towards the structural inclusion of patients.

1.3.2 Research questions

The three sub-questions specified above determined the outline of the study design, which can be divided into three parts as well. The first part of the study focused on the *current situation* concerning patient participation in decision-making on biomedical research agendas. The second part of

[18] The term 'strategy' is used in order to stress the pursuit of a societal objective, namely effective patient participation in decision-making on biomedical research. From a pure scientific perspective, the strategy to be formulated can be considered a new and effective participation methodology.

the study focused on the design of a *new strategy* for effective patient participation. In the third part of the study, subsequently, the proposed strategy was *implemented* and *evaluated* in a concrete practical situation.

In order to answer the three sub-questions, a range of detailed research questions had been addressed following an 'emergent design' (cf. Guba and Lincoln, 1989); each question was formulated in close interaction with the overall objectives of the study and the developing knowledge on the context. Below, the subsequent research questions are described in relation to the main contextual aspects that have influenced their formulation.

A more thorough investigation of the *current situation* comprises investigating current attempts of, and strategies applied for, patient participation. This led to the following three research questions:

1. *To what extent are patients actually involved in biomedical research decision-making?*
2. *What strategies are followed to involve patients in decision-making on biomedical research?*
3. *To what extent can these strategies be considered as realizing effective participation?*

The answers to these questions indicated that currently patients are involved in biomedical research decision-making only rarely and that current strategies followed hardly result in effective patient participation. Apparently, effective patient participation in decision-making on biomedical research is hampered. A subsequent research question therefore addressed the obstacles that hamper effective patient participation in decision-making on biomedical research:

4. *What obstacles hamper effective patient participation in decision-making on biomedical research?*

A main obstacle turned out to be the general adhered presupposition that patients lack the knowledge necessary to contribute anything relevant to decision-making on biomedical research. This presupposition thus contradicts the substantive argument for patient participation in this decision-making, which is considered a very essential one since it refers to the (potential) expert-role of patients. In order to investigate the validity of the substantive argument, the next research questions focused on patients' experience-based knowledge and its potential value for biomedical research:

5. *What are characteristics of patients' experience-based knowledge and what could this knowledge contribute to decision-making on biomedical research?*
6. *Is there any evidence that patients' experience-based knowledge has influenced decision-making on biomedical research agendas?*

The findings within the framework of these questions suggested that patients' experience-based knowledge could in principle contribute to decision-making on biomedical research. However, the utilization and integration of this knowledge into biomedical decision-making processes appeared to be still far from optimal.

The second part of the study therefore focused on the design of a *new strategy* that could successfully involve patients and their experience-based knowledge in decision-making on biomedical research agendas. For that reason, subsequent research questions addressed necessary elements of a strategy for effective participation and knowledge integration:

7. *What procedure could be followed in order to involve patients in, and integrate their experience-based knowledge into, biomedical research decision-making processes?*
8. *What further conditions need to be met for effectively integrating patients' experience-based knowledge with biomedical knowledge into biomedical decision-making?*

Finally, in the third part of the study, the proposed strategy for patient participation in decision-making on biomedical research agendas was *implemented* in a concrete practical situation and the effectiveness of the participation exercise was *evaluated*. The implementation took place in the context of a broader interactive health research agenda-setting project that was commissioned by the Netherlands Asthma Foundation and co-financed by the Netherlands Organization for Health Research and Development (ZonMw). An essential part of this project was the explicit consultation of patients for their research priorities. Research questions addressed during this consultation comprised the capability of patients to participate adequately in biomedical research agenda setting.

9. *To what extent are patients capable of adequately identifying and prioritizing health research topics in general and biomedical research topics in particular?*
10. *To what extent do patients value biomedical research?*

Finally, research questions addressed by the overall patient participation exercise were:

11. *What do patients contribute to (biomedical) research agenda setting?*
12. *To what extent can the strategy proposed and implemented be considered appropriate for realizing effective patient participation?*

1.3.3 Methodology

Since the study aimed both to analyse, and to formulate and develop decision-making processes, it included a variety of methods. The first part of the study, focussing on the current situation, involved mainly descriptive-analytical case study research. The second part of the study, focussing on the development of a new strategy, consists of more 'conceptual' research[19]. The third part of the study, involving the testing and evaluation of the formulated strategy in a practical situation, comprised a social experiment. This section describes the different methods used in these three parts of the study as well as the research team involved and the research relationships with participants established during the study.

Methods

The study covers the period from November 1999 until July 2004. Methods used during the descriptive-analytical part of the study (research questions 1-6) included extensive desk studies, interviews, informal dialogues, and an exploratory workshop, which was held in September 2000. Below I shortly describe the different methods used, including the ways of sampling, data collection and data analysis. Further details can be found within the different chapters.

Desk studies comprised both literature and Internet research, searching for background information about relevant actors and their relationships, as well as for theories on, and examples of, (patient) participation in decision-making processes.

Interviews included both exploratory and explanatory interviews with patients, patient representatives, biomedical researchers, health care professionals, representatives of intermediary organizations such as

[19] With the term 'conceptual research' I refer to a form of designing research that aims to build models or strategies to solve problems.

research councils, research funding agencies, and (socio-cultural) researchers in the field of patient participation. All interviews were semi-structured, thus leaving room for interviewees' narrations while still being able to focus on topics relevant for the various research questions (Saunders et al., 2003). Topics addressed during the interviews included interviewees' perspectives on, and experiences with, (biomedical) research and the (possible) role of patients in this research, the content and the value of patients' experience-based knowledge, examples of patient participation in decision-making on biomedical research, etc. Interviewees were either purposively selected, or selected by using the snowball method (Saunders et al., 2003). While some interviewees were selected based on their (expected) experience with, or possible interest in, patient participation in decision-making on biomedical research, others had been interviewed because of their representation of a relevant stakeholder group. Interviews were either fully transcribed from tape recordings or extensively reported from minutes directly afterwards. Interview reports were always returned to interviewees for validation in order to justify descriptions and interpretations.

Interview data were analysed by both contextualizing and categorizing (cf. Maxwell, 1996). During contextualization, main messages and perspectives of interviewees were estimated by trying to understand their stories as small case studies. In order to investigate general themes more thoroughly, interview data were subsequently categorized. Categories used included issues such as 'previous experience with patient participation in decision-making processes' and 'acknowledgement of the value of patients' experiential knowledge', which were inductively generated during the study (inspired by the grounded theory approach of Strauss and Corbin, 1998). In addition, categorized interview data were often placed in matrices in order to obtain more overview and insight.

The exploratory *workshop* included three semi-structured group discussions between representatives from patients' organizations, health care professionals, medical and biomedical researchers, representatives from intermediary organizations, and a representative of the pharmaceutical industry, and aimed to identify opinions and visions on patient participation in biomedical research. Discussions focused on the current situation concerning patient participation in decision-making on biomedical research as well as the desirability and feasibility of enhancing this patient participation. Workshop data were processed and analysed similar to the interview data described above.

The second part of the study, involving the development of a new strategy, was based on literature research and the analysis and interpretation of earlier findings and experiences. The third part of the study, focussing on the testing of the new strategy, consisted of a social experiment that was conducted and evaluated in the period December 2002 – July 2004.

The *social experiment* comprised the implementation and evaluation of a newly developed patient participation strategy within the context of a health research agenda-setting project commissioned by the Netherlands Asthma Foundation (NAF – 'Nederlands Astma Fonds') and co-financed by the Netherlands Organisation for Health Research and Development (ZonMw). The participation strategy was an adapted version of the *Interactive Learning and Action* (ILA) approach[20]; a methodology aiming at involving marginal stakeholders in decision-making on science and technology, developed by Joske Bunders and Jacqueline Broerse from the Athena Institute of the Vrije Universiteit Amsterdam[21] during the 90s (Broerse and Bunders, 1999; 2000).

The newly developed participation strategy consisted of six phases in which different research tools can be used:

1. a preparation and initiation phase;
2. a consultation phase;
3. a collaboration phase;
4. a prioritization;
5. a specification phase; and
6. an implementation phase.

The research agenda-setting project comprised the first four phases only.

During these phases different methods have been used. In the first phase, data collection took place by means of desk studies and interviews, focusing on the current state of affairs concerning (decision-making on) biomedical research on asthma or COPD[22] and on opportunities for patient participation within this research. Methods used in the consultation phase included interviews, focus groups, a questionnaire, and several homogeneous group discussions. The collaboration phase involved an integration meeting that consisted of three heterogeneous group discussions. In the latter two phases the main focus was on priorities for

[20] previously called the 'Interactive Bottom-Up approach'

[21] Athena Institute for Research on Innovation and Communication in Health and Life Sciences

[22] Chronic Obstructive Pulmonary Disease

research on asthma or COPD. The fourth phase consisted of a written prioritization exercise.

Focus groups were used to gain insight in the experienced problems of asthma/COPD patients that may form a basis for their research priorities. The organization of different focus groups geographically spread across the country had to neutralize local biases. Participants were selected on the basis of their NAF membership and the region they were living in. Although we originally planned to form separate groups of asthma and COPD patients, this became infeasible in practice because in two of the three regions only a few COPD patients were able to participate. Other demographic characteristics were considered irrelevant for segmentation. Each focus group was chaired by an experienced facilitator, who was assisted by a monitor and a minute. The focus group design was standardized and averagely structured (cf. Morgan, 1996) using six pre-established questions and exercises. Issues discussed were personal experiences and problems encountered with asthma or COPD, interpretations of those problems, and ideas for research questions. Exercises involved the prioritization of problems in terms of their urgency to be solved.

Focus group discussions were monitored in order to analyse interactions between participants. In addition they were recorded in handwritten minutes as well as on video and cassette for further analysis. All participants consented to these recordings on conditions of anonymity and deletion after the project. The discussions were analysed by searching the minutes and tape recordings for mentioned causes of, and mutual relations between, identified problems. Summarizing reports of the discussions and its outcomes were sent to all participants for feedback. All problems, causes, and mutual relations mentioned were logically analysed in a so-called 'causal tree' (e.g. Klinkers, 2002).

The *questionnaire* aimed to investigate patients' priorities on (potential) health research topics or directions in a quantitative and representative way. Besides some introductory questions on demographic characteristics, the main part of the questionnaire consisted of prioritization exercises in which respondents are asked to divide a maximum number of points among different items (a variant of the 'budget pie' method, see Mullen, 1999). These items were based on main problems mentioned in the focus groups. The questionnaire was tested beforehand, and after some minor revisions ad randomly sent to members of the NAF and to focus group participants. An accompanying letter and a stamped return envelope were included. The questionnaire results were

analysed in a stratified way in order to identify possible influences of demographic characteristics on research priorities.

Group discussions had been conducted in order to consult scientists and professionals on their research priorities. These discussions had partly an exploratory character, focusing on experienced deficits in knowledge or health care inadequacies as well as on research priorities, and partly a deepening character, focusing on mutual relationships between deficits, arguments for priorities, and consensus on priorities. Participants were selected on the basis of their central position within a discipline, while all scientific and health care disciplines involved with asthma and COPD were sought to be covered. All discussions were recorded in minutes and on tape. Discussion minutes were sent to all participants for feedback. Final analysis included the incorporation of experienced deficits or problems in causal trees and the listing of agreed priorities.

Additional *interviews* aimed at investigating the priorities of relevant stakeholders who had attended group sessions or at gaining more in-depth insight in priorities of stakeholders involved. All interviews were conducted and analysed as the interviews described above.

A subsequent *integration meeting*, including patients, scientists, and health professionals, aimed to discuss perspectives and priorities of different stakeholders, to accomplish mutual respect, understanding, and learning between the different stakeholders, to integrate the different research priorities into one research agenda, and to identify criteria for further prioritization of the research agenda. For this meeting professionals were selected based on their backgrounds and on their indicated willingness to share decision-making with patients. Patients were selected from focus group participants based on their indicated wish to attend an interactive meeting and on their capability to express themselves clearly as assessed during the focus groups.

The meeting consisted of three parallel, heterogeneous group discussions. These discussions were carefully guided by experienced facilitators and recorded in minutes and on tape. Afterwards all participants were interviewed in an in-depth way in order to evaluate the overall process (see below) and to assess their agreement with final outcomes.

The final step in the research agenda-setting project comprised a written *priority setting exercise*. For this purpose a prioritization matrix was sent to all professionals that had participated in the agenda setting process as well as to patients who had indicated to be willing to participate in dialogues with professionals. Vertically in the matrix the different research

topics of the overall health research agenda were listed, while horizontally respondents were asked to rate the different research topics of the agenda by giving marks and to indicate in the matrix which criteria determined their priority setting. Final priorities were determined by the frequencies research topics had been prioritized, as well as by the total scores (added marks) of the different research topics.

Methods used within the *evaluation* of the agenda-setting project comprised (comparative) documentary analysis, observation, video and cassette tape analysis, and interviews with participants.

Comparison between intermediary results of the consultation phase and the end results of the project provided insight in the actual influence of the different stakeholders on the final integrated research agenda. Analysis of feedback reports and evaluation forms provided additional information on participants' opinions concerning the adequacy of the procedures used, the quality of the analysis of results, etc.

(Direct) observation and tape analysis were used to investigate social aspects of the process more thoroughly, such as the equal approach of, and interaction between, participants and their actual inputs in discussions as well as the functioning of the discussion facilitators. Observations were made by both facilitators and monitors. Although observation holds the danger of leading to subjective and biased results, it also provides important additional research opportunities (e.g. personal experience) that might be missed when restricting to more objective and retrospective evaluation methods only. Furthermore, observations do not depend on people's willingness to respond to questions (Taylor-Powell and Steele, 1996).

In-depth interviews with all participants of the integration meeting and two NAF-managers were used to collect visions on both the process and its outcomes more thoroughly. All interviews were held in the period April to June 2004 and carefully recorded. Interview reports were returned to the interviewees for feedback in order to validate any interpretations and conclusions. Interview reports were analysed as described previously.

Research team

During the entire study, the research team involved different people. The major part of the descriptive-analytical study (both interviews and desk studies) was conducted by the author of this book. Thereby I was occasionally assisted by MSc students who did some desk studies or performed some interviews within the framework of a traineeship.

The social experiment was a team effort. Besides me, Jacqueline Broerse and Tjard de Cock Buning of the Athena Institute were involved in the overall design and management of the project and were facilitators during several focus groups and group discussions. A junior researcher, Julia Teerling, was responsible for the main part of the practical management, including the invitation of, and communication with, participants, the organization of meetings, the processing of results, etc. Three MSc students, Melissa van Alst, Simon Klaasen, and Edwin Swart, were involved in the project as trainees for a six-month period and conducted amongst others the evaluation interviews. In addition, a group of six MSc students assisted in the organization and processing of patient focus groups within the framework of a course on interactive research methodologies. This intensive collaboration with both colleagues and students explains the use of the personal pronoun 'we' in the remaining part of this dissertation.

Research relationships

During the study there was intensive interaction with patients, patients' organizations, biomedical researchers, health professionals, policy-makers, and other relevant actors in the fields of patient participation and biomedical research. Since these relationships may have influenced the research results, it is necessary to elaborate on this issue (cf. Maxwell, 1996).

Relationships with the patient community as well as with biomedical researchers had to be built up carefully. Interpersonal aspects such as mutual respect and trust appeared determining for the success of interactive data collection and the quality of data gathered. In time, many patients had become reserved towards researchers because of their experience of not being taken seriously by them. They thus needed to be approached as experts in an open-minded and respectful way. At the same time, some biomedical researchers appeared to feel uncomfortable by the idea of patients interfering in their domains and co-determining research agendas. They as well needed to be treated with respect and with acknowledgement of their autonomy and concerns.

In addition, the choice of words appeared to be very important for the quality of results. For example, during my search for a patient group that was willing to participate in a social experiment, I experienced that it was essential not to introduce myself as a 'researcher' since many patients and patients' organizations have become tired of being involved in

research, as they tend to be overloaded with requests from (clinical or social) researchers that seek their collaboration and information but often fail to communicate the results. However, when speaking to biomedical researchers or health professionals, it was important to present myself clearly as an academic researcher in order to be taken more seriously.

In addition, the explicit focus of this study on biomedical research appeared to be rather restrictive for constructive interaction with patients. Firstly, the term 'biomedical' puts many patients off since they are not familiar with this term. Secondly, patients' perspectives on, and interests in, research usually are much broader than biomedical research only. Therefore for the consultation and participation of patients it was quite convenient that the agenda-setting process in the last part of this study focused on health research instead of on biomedical research (as was also an explicit wish of the NAF and ZonMw).

1.3.4 Validity

Within this study, different strategies have been used in order to enhance the validity of results and conclusions and to minimize effects of researcher bias and influence (Maxwell, 1996). The following strategies will be shortly described: triangulation, member checks, and the use of rich data.

Triangulation

A first methodological strategy that strengthens the validity of this study is triangulation: the exploration of various data sources, with various methods. Triangulation reduces the risk of systematic biases or limitations of a specific method (Maxwell, 1996).

In the descriptive-analytical part of the study, triangulation was achieved by collecting information from literature, websites, as well as from many individuals with different backgrounds and perspectives, respectively through desk studies and interviews.

In the social-experimental part of the study, there was some triangulation during the evaluation of the participation project, by combining different data collection methods such as documentary analysis, (direct) observation, and interviews with a diversity of participants, such as patients, scientists, and health care professionals. Furthermore, since different team members were involved in this evaluation, there was a

triangulation among investigators as well, which further minimizes effects of personal researcher bias or influence.

Member checks

Another strategy that was frequently applied concerns the so-called 'member checks' (Guba and Lincoln, 1989; Maxwell, 1996), consisting of systematically soliciting feedback on data and conclusions from the people who were studied. In this study, after each interview, focus group, or group discussion, interviewees or participants received draft reports in order to check interpretations of opinions or perspectives. In this way misinterpretations or mistaken conclusions through researcher bias or misunderstanding were minimized.

Rich data

An important strategy followed in this study in order to enhance the validity of results was the extensive documentation of primary data, such as authorized verbatim transcripts of interviews.

1.4 Outline of the book

The chapters 2-6 of this dissertation are based on separate articles and a report that have been published or submitted for publication before. They successively address the different research questions described above.

The chapters 2 and 3 comprise the descriptive-analytical part of the study. Chapter 2 focuses on the first four research questions, by elaborating on current strategies for patient participation in biomedical research decision-making and on obstacles that hamper more effective and structural patient participation. At the end of this chapter I reflect on some strategic elements that could contribute to overcoming these obstacles. Subsequently, chapter 3 deals with one of the main obstacles identified by investigating, and reflecting on, the potential value of patients' experience-based knowledge for decision-making on biomedical research, thus addressing research questions 5 and 6.

Chapter 4 involves the second part of the study and addresses research questions 7 and 8. Building on the findings of the first part of the study, literature research, and on earlier experiences with participatory strategies, I discuss the possible design of, and conditions for, an appropriate strategy to involve patients and integrate their experience-

based knowledge within decision-making on biomedical research in an effective way.

Subsequently, the chapters 5 and 6 comprise the social experimental part, describing the implementation and evaluation of the developed strategy within the context of an interactive agenda-setting project concerning research on asthma or COPD. While chapter 5 deals with the consultation of patients on their health research priorities and thus addresses questions 9 and 10, chapter 6 describes the overall participation trajectory and its evaluation, thus addressing questions 11 and 12.

In chapter 7, finally, the main findings that answer both the different research questions and the main research question are presented and discussed. In addition some suggestions for further research are given.

2

CURRENT STRATEGIES AND OBSTACLES[23]

This chapter elaborates on the current situation concerning patient participation in decision-making on biomedical research, and in particular on the situation in the Netherlands. Subsequently, the role of patients in the current biomedical decision-making network, strategies followed to implement patient participation, and obstacles that hamper optimization of this participation are investigated and described. The findings indicate that user participation in decision-making is not a widespread phenomenon in the biomedical field. The examples found suggest that in the Netherlands three main strategies for patient participation can be distinguished. We argue that these strategies concern rather minimal levels of participation, since they do not entail partnership, ensuring patients' structural influence on decision-making. They could be applied because they involve little or no change in the current biomedical decision-making network. In addition, we identified various obstacles that hamper a more effective involvement of patients in decision-making on biomedical research. The majority of these obstacles seem to reflect the resistance of the biomedical decision-making network. In the last section of this chapter the concept of transition management is introduced in search for clues on how to change the network towards more equal partnership between patients and professionals in decision-making processes concerning biomedical research.

As stated in the introduction of this book, patient participation in biomedical research decision-making is rarely described in academic literature. This suggests that patients are largely uninvolved in decision-

[23] The text of this chapter is based on Caron-Flinterman, J.F., Broerse, J.E.W., and Bunders, J.F.G. (submitted). Patient partnership in decision-making on biomedical research: Changing the network. *Science, Technology, & Human Values.*

making on biomedical research. In order to investigate this assumption more thoroughly, we focused on the situation in the Netherlands during the period 2000-03 as a case. For this purpose, this chapter subsequently elaborates on (1) the decision-making network in the Netherlands and the role of patients in this network, (2) the strategies followed for the implementation of patient participation in decision-making on biomedical research, and (3) the obstacles that hamper the implementation of a more effective participation. In the final section of this chapter we discuss a possible route to overcome these obstacles.

Data were collected by means of an exploratory workshop, which was held in September 2000, 61 semi-structured interviews and more than 20 informal dialogues with relevant actors – biomedical scientists, patients, and representatives from 'intermediary organizations'[24] –, all conducted in the period 2000-2003, as well as some additional desk studies. In the workshop five biomedical scientists, four medical doctors, one representative of the pharmaceutical industry, five patients and patient representatives, two representatives of intermediary organizations, and four researchers of health-related inter- or transdisciplinary research fields were invited. In three heterogeneous discussion groups participants discussed the current situation concerning patient participation in decision-making on biomedical research as well as obstacles and opportunities for increasing the involvement of patients in this decision-making. Participants had been selected partly on their possible interest in patient participation and partly on their representation of a relevant organization or professional community.

Informal dialogues aimed at exploring stakeholders' views on patient participation in biomedical research decision-making. Interviews included both exploratory and explanatory, semi-structured interviews with 22 patients and patient representatives, 19 biomedical researchers, 3 health care professionals, and 17 representatives from intermediary organizations. They aimed at investigating more thoroughly stakeholders' roles and interactions within the biomedical research decision-making network, as well as their views on obstacles and strategies for patient participation in this network. Interviewees were selected on the basis of their involvement or interest in biomedical research decision-making and the possible role of patients in this decision-making.

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[24] With the term 'intermediary organizations' we refer to all organizations that are somehow concerned with the interface between science and society, such as governmental research councils, research funding agencies, and social research institutes or departments working on the democratization of science.

Desk studies provided additional information on the structure of the biomedical research decision-making network and on possible routes to implement patient participation within this network.

2.1 Decision-making on biomedical research in the Netherlands

As described in section 1.1, decision-making on biomedical research involves a network of actors that interact in a more or less stabilized manner. Each actor has its own interests, role, and degree of involvement within the network. The most involved actors are, of course, the biomedical researchers, who work at universities, research institutes, or industries, and are the initiators, designers, and executers of individual research projects.

Other key actors are the various biomedical research sponsors, comprising federal funding agencies, charity funds, and industry. The Netherlands Organisation for Health Research and Development (ZonMw) is the largest public research-funding agency involved with biomedical research in the Netherlands. It formulates and executes both basic and applied health research programmes with an annual budget of about € 70 million (ZonMw, 2003). The majority of ZonMw's commissions come from the Ministry of Health, Welfare and Sport (VWS – 'Ministerie van Volksgezondheid, Welzijn en Sport') and the Netherlands Organisation for Scientific Research (NWO – 'Nederlandse Organisatie voor Wetenschapplijk Onderzoek'). The Ministry's main concern is to contribute to public health, including prevention and health care services, by funding applied biomedical and other health research. NWO, as the national research council, is concerned with fundamental and strategic research. In terms of health research and development – and thus in its relation with ZonMw – its main interest lies in contributing to cure, care and prevention by gaining a better understanding of disease and its underlying processes, and supporting research related to medical or health technology assessment.[25]

Examples of large Dutch charity funds involved with biomedical research are the Dutch Diabetes Research Foundation ('Diabetesfonds'), the Netherlands Alzheimer Foundation ('Stichting Alzheimer Nederland'), the Dutch Cancer Society ('KWF kankerbestrijding'), the Netherlands Asthma Foundation ('Nederlands Astma Fonds'), the Netherlands Heart Foundation

[25] www.zonmw.nl

('Hartstichting'), the Dutch Kidney Foundation ('Nierstichting Nederland'), and the Dutch Arthritis and Rheumatism Foundation ('Reumafonds'). In the Netherlands, most charity funds are independent of patients' organizations, although they often do provide support and information to, and sometimes collaborate with, patients and patients' organizations. Decisions on research programmes and fund appraisal are usually taken by scientific advisory committees or programming committees consisting of experts, i.e. renowned biomedical researchers and medical practitioners, only.

At a less centre stage, but still quite influential in decision-making on biomedical research, is the Dutch government. The Dutch Ministry of Education, Culture and Science (OC&W – 'Ministerie van Onderwijs, Cultuur en Wetenschap') regulates, coordinates, and finances academic scientific research in general. It provides both direct (basic) funding to universities and indirect funding via intermediary organizations such as NWO and the Royal Netherlands Academy of Arts and Sciences (KNAW – 'Koninklijke Nederlandse Akademie van Wetenschappen') (Ministry of Education Culture and Science, 2004). In the Netherlands non-allocated governmental contributions to universities forms a major source of research funding, providing the (biomedical) research community a lot of freedom in the choice of research topics and projects (Cabello et al., 1999; Ministry of Education Culture and Science, 2004). In addition, the Ministry of Health, Welfare and Sport (see above) and, at a smaller scale other ministries, such as the Ministry of Economic Affairs fund specific (more applied) biomedical research programmes or projects (Van Hoëvell-van Dapperen, 1998). An influential advisory council is the Advisory Council on Health Research (RGO – 'Raad voor Gezondheidsonderzoek'), which advises the government on national health research priorities and programmes.[26]

Another role of the Dutch government in the biomedical decision-making network is as main client of (semi-) governmental (bio)medical research institutes, such as the National Institute for Public Health and the Environment (RIVM – 'Rijksinstituut voor Volksgezondheid en Milieu') and TNO Prevention and Health, the medical research institute of the Netherlands Organization for Applied Scientific Research ('TNO Preventie en Gezondheid').

Other actors that occasionally influence decision-making on biomedical research in an indirect way, are medical professionals, patients and patients' organizations, supporters of charity funds, and pressure

[26] www.rgo.nl

groups, such as the Dutch Society for the Protection of Animals ('Dierenbescherming') (see section 1.1).

Both the interviews and the literature research confirmed that patients and patients' organizations, although highly involved in the biomedical research field as end-user group, are less influential in terms of decision-making on biomedical research. While at a national policy level, some patients' organizations lobby for certain types of research (e.g. research on genetic therapy), they are not structurally involved in formal decision-making processes. At a project level, patients' roles usually remain restricted to supplying research substrates and being the ultimate target group of the research outcomes. Decision-making on biomedical research agendas, on individual levels as well as on institutional and national levels, is mainly the territory of experts. It resembles a technocratic or expert-model of governance (see also Salomon, 2000). Only at a very limited scale research funding agencies, patients' organizations, and advisory councils experiment with involving patients in decision-making on biomedical research (see below).

2.2 Current strategies for patient participation

Although in general patients are marginally involved in decision-making processes concerning biomedical research in a formal way, in our study we identified various concrete Dutch cases of patient participation in decision-making on biomedical research. Though in all these cases patients or their inputs have been involved in decision-making processes, the cases differ in strategies (implicitly) followed. We found that in the Netherlands, patient participation in biomedical research is mainly achieved in three ways: (1) the successful lobbying of patient organizations, (2) the ad hoc use of patients' ideas and demands through intermediaries, and (3) the inclusion of patient representatives in existing decision-making committees or councils. Below, we describe these strategies and illustrate them by mentioning some concrete examples (see tables 2.1-2.3). Although these examples do not reflect an exhaustive investigation, we think they together give a reasonable impression of the current status quo concerning patient participation in decision-making on biomedical research in the Netherlands.

1) Successful lobby of patients' organizations
The first strategy comprises patient organizations who take the initiative to participate in decision-making on biomedical research. They push forward their demands concerning research priorities by strongly lobbying for the general acknowledgement and subsequent expansion of a certain type of biomedical research. In table 2.1 we shortly describe two examples of this strategy.

Table 2.1 Examples of the successful lobby of patient organi: ations

-	After years of lobbying the Dutch Alliance of Parents' and Patients' (rganisations (VSOP – 'Vereniging Samenwerkende Ouder- en Patiëntenorg nisaties') is increasingly consulted in national and international policy making on genetic research. It has also established seven academic chairs on topics su h as clinical genetics and rare diseases.
-	During the past ten years the Dutch Society of Haemophilia Patien s (NVHP – 'Nederlandse Vereniging van Hemofilie-Patiënten'), alongside o her patient organizations, has successfully lobbied for more governmental suppor for research on genetic therapy.

More examples of this way of influencing biomedical research policy exist, both on national and international level. They mainly concern the lobby for genetic research. Genetic research is generally expected to give rise to important breakthroughs concerning the cure of genetic illnesses and is therefore strongly advocated by many patient organizations concerned with genetic diseases. At the same time, patient organizations feel the need to influence policymaking on genetic research since this type of research has several controversial and complicating aspects that may delay governmental permission of its adoption.

According to several respondents, in the cases identified the strategy of lobbying is successful due to the professionalism and perseverance of the patient organizations involved. Although these patient organizations are not actually included in (formal) biomedical decision-making structures, they increasingly are considered stakeholders that should be taken into account in national or international policy making.

2) Ad hoc use of patients' ideas and demands through intermediaries
In the second strategy individual professionals or organizations, such as medical researchers, science shops, or research funding agencies, take the initiative to involve patients' inputs in decision-making on biomedical

research programmes or projects. They ask for and/or take up patients' demands or ideas and translate these into questions, topics or priorities that fit into current biomedical research practices, thus acting as intermediaries between the patient community and the research community. In table 2.2 we shortly describe four examples that reflect this strategy.

Table 2.2 Examples of the ad hoc use of patients' ideas and dem nds through intermediaries

- In consequence of many complaints and questions from p tients with neuromuscular diseases about the severe fatigue they suffered fro , a medical specialist of the Neuromuscular Centre Nijmegen (University Me lical Centre Nijmegen) launched a new research project on central and peripher l aspects of muscular fatigue.
- The Dutch Addison and Cushing Society (NVACP – 'Nederlands Vereniging voor Addison en Cushing Patiënten') approached a science shop in Utrecht with the request for a study on drug administration methods in order t search for improved medication of Addison's disease. The study was executed b a pharmacy student. The professor that had supervised the study subsequently stablished a research project on a new delayed release tablet.
- After consulting the members of the Dutch Kidney Patient Organisa on (NVN – 'Nierpatiënten Vereniging Nederland') on the relevance of the subject ia an article in their magazine, a biomedical researcher launched a research project n causes of, and therapies for, 'restless legs' as a symptom of kidney disorders. The study is funded by the Dutch Kidney Foundation ('Nierstichting Nederland').
- A programming committee of the Netherlands Organisation for Scient fic Research (NWO – 'Nederlandse Organisatie voor Wetenschappelijk Onderzoe ') consulted the United Patients' Organisations of the Chronically Ill (WOCZ – ' Verkverband Organisaties Chronisch Zieken') for formulating a research priority fo a remaining part of an integral research programme on chronic illnesses. After co sultation of its grassroots, the WOCZ introduced research on co-morbidity a a research priority, which was adopted.

In our study most interviewees stated that mediation by intermediaries is essential for introducing patients' ideas and demands into the research field:

> "An intermediary needs to translate individual patient questions into more general and researchable questions. Such an intermediary thus has to be familiar with both research and patient worlds" (a representative of an intermediary organization).

At the same time intermediaries can help to distinguish useful from not useful ideas and demands, for example by searching for inter-subjectivity or by estimating scientific tenability.

3) Patients' membership of decision-making committees or councils

The third strategy comprises the involvement of patient representatives in formal decision-making structures, such as national or institutional committees or councils that advice or decide on research policy, programmes, or priorities. In table 2.3 we give four examples of this strategy. In the examples described, initiators were either the government or research funding agencies, which stimulated, required, or even realized patient participation in decision-making structures.

Table 2.3 Examples of the inclusion of patients in decision-makin committees or councils
- The Advisory Council on Health Research (RGO – Raad voor Gezondheidsonderzoek') in the Netherlands, that advises the gov ernment on national research programmes and priorities, has one patient mem ber. Also in temporary programming and priority setting subcommittees, patients a e involved.
- In response to a governmental request to involve patients as much a possible in research decision-making, the societal advisory council and several programme committees of the Netherlands Organisation for Health R search and Development ('ZonMw') now have several patient members.
- The Rheumatism Foundation ('Reumafonds') in the Netherlands has wo patients participating in the scientific advisory council that appraises research roposals on both scientific quality and societal relevance and advises the b ard of the Foundation about financing research.
- Four representatives of the Dutch Neuromuscular Diseases Associa on (VSN – 'Vereniging Spierziekten Nederland') participate in the board of the Dutch Neuromuscular Research Foundation (SONMZ – 'Stichting Onderzoek Neuromusculaire Ziekten') that aims to stimulate and communic te scientific research on causes of, and therapies for, neuromuscular diseases.

Respondents involved within this strategy stressed that proto-professionalization[27] of the patient participants is a prerequisite for successful participation in biomedical research structures that are dominated

[27] The concept of proto-professionalism refers to the degree lay-people have internalized the thinking of a certain professional group and organize, experience, and express their daily lifes according to this thinking. Although this concept originally has been applied within the context of health care, referring to patients that have internalized medical technical insights, which causes them to redefine their own health and illness (De Swaan, 1979), in this chapter we use the concept of proto-professionalism to refer to patients' internalization of biomedical scientific language and principles.

by scientists. In a process of proto-professionalization, patients learn about research procedures and principles and get used to scientific language and thinking. They thus adapt themselves to current biomedical research practices.

2.3 A reflection on current strategies

For the further characterization of the three strategies for patient participation described above, we use the 'Ladder of Citizen Participation' of Sherry Arnstein (1969) as a framework (see section 1.3). According to Arnstein, true participation should at least reflect a level of partnership, referring to some power sharing between patients and professionals.

Within this framework the first two strategies for patient participation can be considered consultation. Patients or patients' organizations either push forward their views (enforced consultation) or are consulted on their views by intermediaries. In both strategies professionals eventually decide about using these views. In addition, these forms of consultation usually occur in a rather ad hoc and non-structural manner.

The inclusion of patients themselves in formal decision-making structures, such as in committees or councils, implies a higher participation level of at least placation or even partnership, depending on the actual and structural influence of patients in decision-making processes[28]. However, most interviewees stressed that this kind of participation usually takes the form of placation rather than partnership, which was criticized by many of them. Only strongly proto-professionalized patients are regarded and treated as real partners by professionals. As one of the involved patient representatives stressed, lack of professionalism easily results in the premature resignation of patients from committees:

> "Unfortunately 4 out of 5 patient participants give up prematurely because they do not manage; they don't speak the language, focus too much on their own illness and don't feel taken seriously" (a patient representative).

[28] In the literature on patient participation, one often refers to this strategy by using the term 'collaboration' (e.g. Boote et al., 2002; Hanley et al., 2000; Oliver et al., 2004; Telford et al., 2002; Williamson, 2001). Although this collaboration is usually defined as 'active partnership', in practice it also comprises forms of placation since the actual influence of patients on research agendas usually remains obscure (see Oliver et al., 2004: 88).

At the same time, some respondents questioned the representativeness of strongly proto-professionalized patients and thus of their inputs in decision-making. Proto-professionalism easily leads to the loss of patient perspectives (Abelson et al., 2003; Mullen, 2000).

> "Patients, who are very active and often surf the Internet sometimes know more about their disease than their medical specialist. However, such patients usually no longer talk from the perspective of patients" (a representative of an intermediary organization).

But do these strategies result in effective patient participation in decision-making in biomedical research? The term 'effectiveness' refers to the degree intended effects or objectives are achieved. In correspondence to the participation objectives defined in section 1.2.2 we consider participation in decision-making effective if it enhances both the legitimacy and rationality of the decision-making process and improves the quality of decision-making outcomes. This in turn will enhance the social acceptance of the outcomes. Eventually, effective participation will result in enhanced human and social capital as well. Based on similar objectives, scholars dealing with participation in science and technology decision-making state that effective participation should accomplish a form of partnership, entailing the direct and early involvement of patients in fair decision-making processes that include negotiation, deliberation and power-sharing. At the same time effective participation should involve the structural integration of participants' knowledge in, and thus their actual influence on, process outcomes (e.g. Abelson et al., 2003; Fiorino, 1990; Laird, 1993; Rowe and Frewer, 2000; Salomon, 2000; Webler and Tuler, 2000).

The three strategies described above cannot be considered as realizing effective participation in this respect. The first two strategies result in some actual influence of patients on biomedical research agendas but only in an ad hoc manner without realizing any partnership, while the third strategy structurally involves patients in decision-making but cannot warrant their actual influence on the outcomes.

There are, however, strategies for user participation being applied in other research fields that have successfully realized partnerships between professionals and non-professionals in decision-making. Well-known examples concern participatory research within the context of development studies (see for example Broerse, 1998; Cornwall and Jewkes, 1995; De Koning and Martin, 1996) and participatory approaches within the fields of sustainability research (Kasemir, Jaeger et al., 2003; Tress and Tress, 2003) and environmental research (Johnson et al., 2003; Till and Meyer, 2001).

Since we did not come across examples of the application of such strategies within the framework of biomedical research, we asked our respondents what reasons they could give for this observation.

2.4 Obstacles to patient partnership

The interviewees mentioned a number of obstacles that hamper true partnership. These obstacles are related to dominant practices and cultures within biomedical decision-making structures as well as to characteristics of patients that are to be involved in those structures. Below we describe the main obstacles identified, illustrated by some quotations[29] of interviewees.

Sense of urgency

Maybe the most obvious 'obstacle' is that not all actors do consider it necessary to change the current situation and involve patients in decision-making on biomedical research. Referring to the many biomedical successes of the last century, achieved without any interference of patients, some interviewees did not see any surplus value of patient participation in decision-making on biomedical research. On the contrary, they argued that patient participation only would delay and complicate efficient decision-making processes.

Financing structures and procedures

More practical obstacles mentioned refer to the current research financing structures and procedures. Financing structures and procedures are dominated by academics and are almost exclusively based on scientific criteria. Patient relevance is rarely an important criterion, and the active involvement of patients is rarely propagated:

> "The scientific board consists of scientists only. […] In itself patients' input could mean something but since the appraisal of research project proposals mainly concerns the scientific content it is not useful to involve patients in this process." (a biomedical researcher who is member of the scientific board of a research fund)

Patient participation in decision-making on biomedical research topics or questions is also hampered by the fact that biomedical research funding

[29] The original quotations were in Dutch and were translated by the author.

agencies do not provide extra means in terms of money or time for the involvement of patients.

Characteristics of the biomedical research community

Other obstacles brought forward by several interviewees concern the structure of the biomedical research community, reflected in the presence of more or less specialized national research schools, intra- or inter-university research institutes, research departments, specific research organizations, etc., which links individual researchers to national and institutional research lines and minimizes the room for researchers to take up other research topics that might be relevant to patients. This structure reflects trends of reductionism and specialization within the biomedical research community. As one patient representative noted:

> "The problem of patient participation […] is to be found in the fact that medical specialists have a rather reductionist way of thinking. They only deal with their own, often narrow discipline and are blind to the broader context. Patients, on the other hand, argue just starting from that context. For them the biography is the point of departure."

Reductionism and specialization are regarded as holding back the attention for disease-transcending research topics, such as co-morbidity or fatigue (often mentioned as topics of high relevance to patients). In addition, the award structure within the biomedical research community was often mentioned as an obstacle to patient participation in biomedical research. Researchers tend to prefer dealing with research topics and questions that ensure scientific acknowledgement and increase publication rates, thus improving their career perspectives, to dealing with topics that might be relevant for the patient community.

At a more implicit and cultural level, interviewees designated shared views, norms, and values among professionals as serious obstacles to patient participation in biomedical research. Generally adhered scientific paradigms that determine thinking on science and knowledge, were said to hamper the inclusion of patients and their experiential knowledge into decision-making on biomedical research at all levels (both policy and project management).

> "If you want to involve patients in research, you have to counter the current scientific paradigm as well as prejudices and patterns of acting and thinking of individual professionals […]. Professionals ascribe a certain position to patients: patients may say how they want to be treated but may not interfere in decisions. They do not possess the necessary knowledge to say anything relevant about [research]." (an intermediary person working on patient participation in mental care research)

Furthermore, researchers interviewed attach great value to their autonomy and stressed the importance of following scientific curiosity:

> "One of the dangers of patient participation in research decision-making is the loss of basic scientifically high-qualified research projects. Basic research, originating from the wish to satisfy scientific curiosity, is very essential. It has proven to be a main source of very important breakthroughs in knowledge development and innovations." (a biomedical scientist)

This scientific curiosity, however, does not necessarily correspond with patients' interests. A patient representative said the following about this:

> "One of the problems of biomedical research is that some research questions might be relevant from a scientific perspective, but these don't have to be relevant at all from a patient's perspective within the framework of his diagnosis or therapy. One gratifies scientific curiosity and produces knowledge but one hardly looks at the interest of the patient."

Characteristics of the patient community

A last category of obstacles that were mentioned by several interviewees refer to characteristics of the patients that are to participate in biomedical decision-making structures, such as their interests, knowledge, and competencies. Firstly, very few patients were said to be willing and able to formulate demands and ideas for biomedical research:

> "Among patients only now and then the wish exists of thinking along with biomedical research. Nor can you usually say that [individual] patients have explicit ideas or demands regarding biomedical research. At a managerial level there will be many more ideas with respect to scientific research, since at that level most questions of patients collect." (a patient representative)

Secondly, various interviewees stressed that most patients lack the knowledge that is considered crucial for adequate participation in decision-making on biomedical research:

> "Patients usually lack the detailed professional knowledge to put their question in a scientific relevant context." (a biomedical scientist)

In addition, patients were said to lack the required objectivity and level of abstraction:

> "Many patients are pre-occupied with their own disease, bring that subject up repeatedly and are not able to look somewhat 'broader'." (a patient representative)

Finally, lack of self-confidence and empowerment, which is considered a prerequisite for adequate communication with professionals by most interviewees, was mentioned as an important obstacle. In table 2.4 the different obstacles are summarized.

Table 2.4 Obstacles to patient partnership in biomedical research decision-making

Sense of urgency
- no observed surplus value of patient participation
- fear for delay and complication of decision-making processes

Financing structures and procedures
- financing structures dominated by academics
- financing procedures based on scientific criteria
- no additional means for patient involvement

Characteristics of the biomedical research community
- strong specialization of research community
- importance of scientific achievements and publication rates
- importance of scientific autonomy and curiosity
- undervaluation of patients' experiential knowledge

Characteristics of the patient community
- limited will to participate
- lack of scientific knowledge
- limited ability of objectification and abstraction
- lack of self-confidence and empowerment

Scholars who have analysed obstacles to stakeholder involvement – mainly from the field of participatory research – largely confirm our findings. They refer to limited resources as well as structural and cultural characteristics of the scientific community as possible obstacles towards effective lay (e.g. patient) involvement in decision-making. Limited resources usually concern time, money, and knowledge (Graham et al., 2001; Gray et al., 2000; Israel et al., 1998). Structural barriers mentioned refer to strong institutionalization and stabilized procedures that hampers any change towards embracing interactive approaches (e.g. Bunders and Van Eijndhoven, 1987; Gaventa, 1998; Gregory, 2000; Israel et al., 1998). Cultural barriers refer to generally accepted values, views, and attitudes of professionals that hamper a successful implementation of interactive approaches (e.g. Cozzens and Woodhouse, 1995; Gray et al., 2000; Israel et

al., 1998). In addition, inappropriate characteristics of the 'lay' community have been mentioned as obstacles to its effective involvement in participatory processes (Boote et al., 2002; Gray et al., 2000; Gregory, 2000; Israel et al., 1998).

The different obstacles together seem to reflect a kind of resistance of the current biomedical research decision-making network towards the structural involvement of patients. This network consists of a more or less fixed number of actors that interact in a stabilized manner, sharing scientific paradigms and following standardized procedures. The whole of shared and stabilized rules and practices within a network is often designated a 'regime' (Geels and Kemp, 2000; Rip and Kemp, 1998). Obstacles attributed to characteristics of the patient community mainly refer to incongruence with the regime of the network. The resistance of networks and regimes towards change has been described before within the framework of socio-technical network theories (Callon, 1991; 1995; Elzen et al., 1996; Geels and Kemp, 2000; Grin et al., 2003; Rip and Kemp, 1998). Actors involved in a socio-technical network tend to adapt themselves to each other and to standardize or normalize their interactions. Changes of interactions within a network, for example by involving new actors, often are felt as destabilizing 'threats' and therefore countered by actors involved (Callon, 1991; 1995; Elzen et al., 1996).

2.5 How to proceed?

The results described above seem to imply that the three identified strategies for patient participation in biomedical research are relatively easily applicable because they largely leave the current biomedical research decision-making network with its dominant regime intact and thus hardly encounter aforementioned obstacles. Actors in the network hold on to familiar ways of thinking and acting, while patients have to adapt themselves to these ways of thinking and acting or to call in an adequate intermediary in order to be heard. This precludes equal partnership, as is clearly phrased by one of the interviewed patients:

> "All 'rules of the game', the objectives, composition and activities [of the committee] had been determined before patients were involved at all. Now the situation has been created that patients are 'allowed to join' while the scientists themselves have decided long ago on what counts as science and good research, what is interesting, etc. Good and effective

> participation is possible only if you have decided or re-decided upon the objective and presuppositions together." (a patient member of a national research council)

Making patients equal partners in decision-making processes concerning biomedical research, ensuring that patients' knowledge is used in a more structural way, implies a rather radical change of the current decision-making network and its regime. Because of the resistance of the network described above, such a change may not be uncomplicated. The question we try to answer in this final section of the chapter is how such a change might be induced and which actor could take the initiative?

For this purpose we searched the literature on socio-technical networks for some clues on how the resistance of networks could be breached and considerable changes could be induced. We found that in particular the rather new field of transition management may provide some interesting insights on this matter. Rotmans et al. (2000: 19) define a transition as "a gradual process of societal change in which society or an important subsystem of society structurally changes". Such a change comprises economic, cultural, technological, institutional, and environmental changes. We regard the stabilized biomedical research decision-making network as a societal subsystem (or subsystem of a subsystem). Changing this network towards a structural involvement of patients in decision-making processes can be considered a transition that includes changing dominant cultures, stabilized patterns of interaction, usual practices, and established institutions. Transition management is about how to manage or induce such a transition and thus might provide some clues on how to realize structural patient participation in decision-making on biomedical research.

Scholars in the field of transition management describe transitions as multi-phase and multi-level processes. The multi-phase concept distinguishes four different stages in the long-term transition process (Kemp and Loorbach, 2003; Rotmans, 2003; Rotmans et al., 2000):

1. a *predevelopment phase*, in which the status quo does not visibly change;
2. a *take-off phase*, in which the process of societal change makes a start;
3. an *acceleration phase*, in which visible structural changes take place through an accumulation of socio-cultural, economic, technological, environmental, and institutional changes that influence each other. The acceleration phase includes processes of collective learning, diffusion, and embedding;
4. a *stabilization phase*, in which the speed of social change decreases and a new dynamic equilibrium is reached.

The multi-level concept distinguishes three levels of social organization that are involved in transitions (Berkhout et al., 2003; Geels and Kemp, 2000; Rotmans, 2003; Rotmans et al., 2001):

1. the *micro level* concerns individual actors and their actions and practices that may develop 'niches' in which technological, social, or policy innovations can arise.
2. the *meso level* is constituted of networks of actors that have shared assumptions and interact via dominant practices and rules ('regimes').
3. the *macro level*, finally, shapes the broad context for niches and regimes, consisting of material infrastructure, the macro economy, demography and the natural environment as well as shared cultures, worldviews, values and paradigms, often referred to as 'landscapes'.

Changing the biomedical research decision-making network and its 'regime' implies changing the meso level. However, in accordance with network theories mentioned earlier (Callon, 1991; 1995; Elzen et al., 1996), the meso level is ascribed considerable resistance against change due to the tendency of existing networks to stabilize and to hold on to current regimes. Therefore in the predevelopment phase, regimes at the meso level act as an inhibiting factor to transitions (Geels and Kemp, 2000; Grin et al., 2003; Rotmans, 2003).

At the same time changes at the macro level or at the micro level can disturb the equilibrium and exert pressure on the regimes, thus inducing a transition (Geels and Kemp, 2000; Rotmans, 2003; Rotmans et al., 2001). Changes at the macro level comprise relatively slow political or societal trends and developments that can play a role in speeding up or slowing down a transition. Changes at the micro level concern the development of innovations within niches, such as new technologies, new initiatives, and new forms of policy, which could gradually transform dominant networks and their regimes in a bottom-up way. Only when changes coincide at all three levels, a transition may occur (Berkhout et al., 2003; Rotmans, 2003).

Concerning a transition towards structural patient participation in decision-making on biomedical research, changes are visible both at the macro and at the micro level. At the macro level trends towards democratization of science and patient empowerment, as we described in the introduction, play a stimulating role. At the micro level, individual actors experiment on involving patients in decision-making on biomedical research programmes, priorities, or topics, as has been the case in some examples described in table 2.1. When successful, these initiatives can be considered

possible inducers of a transition. These changes at both levels suggest that we might be near the start of a take-off phase of a transition towards patient participation in decision-making on biomedical research.

But what can we contribute to this process? A premise of transition management is that although transitions cannot be controlled, they can be influenced. Transition management aims at influencing speed and direction of transition processes. It creates the necessary conditions for societal change by taking the right initiatives at the right time (Rotmans, 2003: 51). Thereby it "joins in with ongoing dynamics and facilitates and builds on bottom-up initiatives" (Kemp and Loorbach, 2003: 11).

Rotmans (Rotmans, 2003) describes a kind of step-by-step guide for transition management comprising four main activities:

1. the design, organization, and management of a 'transition arena' – a small innovation network of actors that are selected on the basis of their competences, interests, and backgrounds, and that form the leaders of the transition;
2. the development of a long-term vision concerning the transition targets and pathways;
3. the steering at learning processes and knowledge production via the planning and execution of innovation experiments; and
4. the monitoring and evaluation of the transition process.

The idea of forming a small innovation network as an initial step in inducing a transition corresponds with one of the conclusions of Elzen et al. (1996) that the establishment of a new socio-technical network, besides the old network, is the most suitable way to bring about radical socio-technical change. These participants all need to share a commitment to the overall targets of the transition. Within the transition arena, there must be enough room for experimentation and innovation on methods for patient participation. At the same time processes of constructive interaction and mutual learning between the different actors need to be facilitated and stimulated.

According to Elzen et al. (1996: 133) the driving force behind the emergence of a new network is one or more so-called 'dedicated network builders', who have a sense of urgency and are prepared to "work against the odds". However, the role of 'dedicated network builders' is a very complex and delicate one. Firstly, they need to have enough power of persuasion to detach other actors (partly) from their stabilized regimes in the old network and to motivate them to participate in a new network. Secondly, they should be intermediaries that are capable of bridging cultural gaps between the

actors and stimulate and facilitate processes of constructive interaction and experimentation. In other words they have to counter the resistance of the current network and overcome the obstacles identified. Since these conditions are difficult to meet, network builders easily fail in their actions. As an example, a small Dutch knowledge agency on research for patients' organizations that was established in 2001 with one of its aims to enhance patients' influence in decision-making on research processes was dissolved recently because of lack of visible success[30].

Inducing the take-off phase of a transition towards patient participation in decision-making on biomedical research could thus entail the creation of a small innovation network (a 'transition arena') including, for example, some interested and open-minded biomedical researchers, patients, intermediary persons, and representatives from research funding agencies and governmental organizations, but also experts from the field of participatory approaches. Actors involved need to acknowledge the possibilities and benefits of patient participation in biomedical research and be willing to experiment with participation strategies. Potentially successful dedicated network builders could be intermediary organizations or academic institutions that have made network building, transition management, or interactive approaches their core activity. Also charity funds that implement their own research programmes could be in a position to fulfil this role. Since these funds usually have close relations with both a patient community and a research community, they might be able to influence both parties and to bridge cultural and structural gaps effectively[31]. Interviews with representatives of the Dutch Kidney Foundation, the Dutch Arthritis and Rheumatism Foundation (RF), and the Netherlands Asthma Foundation (NAF) have shown that these charity funds seek to legitimize their funding practices by involving patients in decision-making. As a result, both the RF and the NAF are already taking initial steps towards patient participation in research programming and prioritizing. In a subsequent acceleration phase

[30] Stichting de PatiëntenPraktijk; www.patientenpraktijk.nl

[31] Although in the Netherlands most charity funds and patient organizations constitute separate organizations, in many other countries, patient associations combine the functions of research fund and patient society. This combination of functions may facilitate patient involvement in decision-making on research programmes or priorities, in particular if patients are on the board of the organization. Two successful examples of patient associations that structurally influence research agendas in this way are the Alzheimer's Society in the United Kingdom (Alzheimer's Society, 2002) and the French Muscular Dystrophy Association (AFM – 'Association Française contre les Myopathies'; see box 1.2) (Rabeharisoa and Callon, 2002; 2004).

the small innovation network could, by establishing new and successful participation practices (such as the launching of an appropriate strategy for participatory programming of biomedical research that leads to relevant and societal embedded research programmes), convince and include ever-more actors out of the old network, thus gradually substituting the current biomedical research decision-making network.

2.6 Concluding remarks

In this chapter we reflected on the current situation concerning patient participation in decision-making on biomedical research. Thereby we focused on the situation in the Netherlands. In spite of possible differences concerning specific situations in other countries, informal communication and additional literature research have indicated that most findings and conclusions are broadly applicable (e.g. Boote et al., 2002; Kent, 2002; Oliver et al., 2004). Although different initiatives indicate that actors experiment with patient participation, we found that the implementation of patient participation in a structural and effective way is hampered by the resistance of the current biomedical decision-making network and its regime. Structural and effective patient participation in decision-making on biomedical research requires a more radical change of this network, which could be considered a transition.

Transition management thus may offer a way to breach the resistance of the biomedical research decision-making network and to realize a change of the network towards patient participation. Thereby one could join in with ongoing trends of user participation in science and build on current bottom-up initiatives identified above. A possible outcome is the emergence of a new biomedical research decision-making network that structurally includes patients as partners in decision-making processes.

A precondition for a successful transition, however, is that patient participation really and visibly contributes to the quality and relevance of research and research agendas. This would support the substantive argument for patient participation in decision-making on biomedical research. For this purpose additional research should focus on the potential contribution of patients to decision-making on biomedical research. Therefore we elaborate on this issue explicitly in the next chapter.

3

THE EXPERIENTIAL KNOWLEDGE OF PATIENTS[32]

One of the arguments in favour of patient participation in decision-making on biomedical research concerns the contribution that patients could make to the relevance and quality of biomedical research based on their 'experiential knowledge'. This chapter reflects on the validity of patients' experiential knowledge in the context of biomedical research processes. Since a conclusive argument on the validity of patients' experiential knowledge could not be reached on the basis of only theoretical reflection, a pragmatic approach was chosen that assessed this validity in terms of its practical usefulness for biomedical research. Twenty-three cases of patient participation in biomedical research were identified and analysed for a concrete contribution of patients to the research process. In nine of these cases, the concrete use of patients' experiential knowledge could be traced. The findings suggest that patients' experiential knowledge, when translated into explicit demands, ideas, or judgements, can contribute to the relevance and quality of biomedical research. However, its deliberate use would require additional research on a more structural and interactive approach to patient participation.

In the introduction of this book, different arguments for involving patients in decision-making on biomedical research have been mentioned. Normative arguments can refer to the moral right of patients to participate in decisions that may substantially affect their lives and bodies, as well as

[32] The text of this chapter is based on: Caron-Flinterman, J.F., Broerse, J.E.W., and Bunders, J.F.G. (2005). The experiential knowledge of patients: a new resource for biomedical research? *Social Science & Medicine* 60: 2572-2584.

to democratic ideals that plead for the participation of stakeholders in decision-making on science in general. Instrumental arguments usually refer to the increased social acceptance of biomedical research directions and outcomes when stakeholders (and thus patients) are involved in decision-making processes. Substantive arguments, finally, refer to the contribution patients could make to the quality and relevance of biomedical research, especially through the specific kind of knowledge and expertise that patients gain as a result of experiences with their illness. This knowledge could complement the knowledge of researchers by providing wider perspectives and options (Entwistle et al., 1998; Goodare and Lockwood, 1999; Popay and Williams, 1996).

This last type of argument is of specific importance since it refers to the (potential) expert status of patients within biomedical research decision-making, which distinguishes patients from other stakeholders such as the public at large. However, at the same time this type of argument is debated. Given its technical character, decision-making on biomedical research is generally considered to require highly specialist knowledge, which makes it a less obvious option for patient participation. As has been shown in the previous chapter, both biomedical researchers and patients themselves often argue that patients lack the knowledge and objectivity that would enable them to make any relevant substantive contribution to biomedical research processes, which is clearly illustrated by the remark of a biomedical researcher we interviewed:

> "Patients should not interfere in processes of which they know nothing about."

This opinion, also observed by Boote et al. (2002) and Oliver et al. (2001), constitutes one of the main obstacles that hamper the implementation of effective patient participation in decision-making on biomedical research.

However, others argue in line with the substantive argument mentioned above that the specific knowledge of patients is a rich source of information that should not be missed in biomedical research, and that the fact that it may be difficult and complex to realize is a poor excuse for not pursuing the integration of patient knowledge into biomedical research processes (cf. Entwistle et al., 1998; Flinterman et al., 2001). One patient whom we interviewed, a member of several consumer-oriented and patient-oriented organizations, remarked:

> "Biomedical science is very reductionist. This leads to useful knowledge and innovation, but the broader context – the overarching 'system' – is ignored. Patients have specific knowledge about what it is like to live

> with one or more ailments. By not involving patients, biomedical research is overlooking an important source of knowledge."

These contrasting views call for closer scrutiny.

This chapter therefore focuses on the added value of patient participation for biomedical research: what knowledge can patients contribute to the biomedical research process? After a theoretical reflection we investigate the validity of patients' knowledge by analysing its potential value for biomedical research in practical examples. To this end interviews were conducted with more than 60 (bio)medical scientists, patients, representatives from patients' organizations, and professionals from intermediate organizations, such as research councils, research financiers, research institutes focusing on patient empowerment or patient participation, research knowledge agencies for patients and patients' organizations, etc. The practical feasibility of structurally including patients in biomedical innovation processes is discussed in the final reflection.

3.1 A theoretical reflection

Expert knowledge is usually considered more general and objective and therefore more accurate than the subjective knowledge of lay persons – which some authors call 'lay' or 'non-expert' knowledge (Entwistle et al., 1998; Nordin, 2000; Popay and Williams, 1996). To avoid any suggestion of inferiority we use the term *'experiential knowledge'* which directly refers to the ultimate source of patient-specific knowledge – the often implicit, lived experiences of individual patients with their bodies and their illnesses as well as with care and cure. Experiential knowledge arises when these experiences are converted, consciously or unconsciously, into a personal insight that enables a patient to cope with individual illness and disability. When patients share experiential knowledge the communal body of knowledge exceeds the boundaries of individual experiences. This body of knowledge has been described as *'experiential expertise'* (Meijer et al., 1993; Van der Schaaf and Oderwald, 1999). Both experiential knowledge and expertise of patients can be extended by scientific (bio)medical insights, for example by reading scientific articles or by discussion with professionals, leading to so-called *'proto-professionalism'*.[33]

[33] As has been remarked before (section 2.3) this proto-professionalism at the same time easily leads to the loss of original perspectives and thus of experiential knowledge.

Whether experiential knowledge can be considered 'valid knowledge' depends on the definitions of both 'knowledge' and 'validity'. Knowledge can be (1) propositional knowledge, (2) procedural knowledge, and (3) knowledge by acquaintance (Lehrer, 1990). Propositional knowledge – 'knowing that' ('smoke worsens asthmatic symptoms') – is the information part of knowledge, made explicit and communicable through speech or writing. Procedural or practical knowledge – 'knowing how' ('to use an inhaler correctly') – is the competence part of knowledge and consists of skills and capacities. It is partly implicit and must be acquired by training and practice. Finally, knowledge by acquaintance – 'knowing' as being familiar with ('what it is like to have an asthma attack') – is implicit knowledge that must be acquired by personal or even bodily experience. Although in practice the three types of knowledge are closely intertwined, below we analyse both experiential knowledge of patients and biomedical knowledge of researchers in terms of these three types of knowledge in order to be able to compare them more clearly.

The specific, experiential knowledge of patients emerges when patients acquire some knowledge by acquaintance through becoming familiar with their own body and illness, with care and cure and with their social context. Subsequently patients develop some practical knowledge, mainly consisting of physical and mental coping strategies. This type of knowledge is important in daily practice, both in the patient's own life and in the support of others. Only after they have made repeated observations and experiences explicit and have reflected on them, can patients acquire some propositional experiential knowledge about the functioning of their bodies, the occurrence of symptoms, the effectiveness of certain therapies, etc. This knowledge is confirmed and extended in the repetition of their own experience, and by similar experiences of other patients.

The most obvious and probably most basic part of the biomedical knowledge of scientists is propositional knowledge, which is obtained both by written and oral knowledge transfer from external sources and by personal knowledge acquisition through experimentation, observation, or argumentation. It begins to emerge during the first years of academic study. Biomedical knowledge also comprises practical knowledge and knowledge by acquaintance, both acquired in practical training and professional practice and both essential to the practice of biomedical research. All three types of knowledge contribute to decision-making in biomedical research, but they are not equally explicit.

Thus, both experiential knowledge of patients and biomedical knowledge of scientists comprise the same three types of knowledge, but

their distribution and order of genesis differ. Furthermore, biomedical knowledge concerns external objects and is mainly acquired through detached and impersonal study and observation, while patients' experiential knowledge concerns the personal situation and is acquired through personal and bodily experiences. In this sense the experiential knowledge of patients can be said to complement the biomedical knowledge of professionals (see also Entwistle et al., 1998; Goodare and Lockwood, 1999; Popay and Williams, 1996).

The issue of validity of knowledge belongs to the domain of epistemology, which traditionally focuses on propositional knowledge only. Within that framework, perspectives on the validity of patients' experiential knowledge depend on the paradigm adhered to. Since Plato, many movements and schools within philosophy have debated the issue of knowledge and its validity. A still rather influential epistemological movement dating from the first half of the 20th century is logical positivism or logical empiricism. Logical positivists argue that the only source of true knowledge is objective observation and that derived knowledge has to be based on rational arguments that follow a logical scheme (Carnap, 1966). Similarly, many scholars since then have considered scientific knowledge an ideal form of knowledge, as it is built on objective scientific methodologies and rational arguments, and strives for universality and absolute truth. They may deny the validity of the experiential knowledge of patients because of its lack of objectivity, verifiability, universality, or rationality. These views also largely determine (and explain) the opinion of many biomedical scientists on the knowledge of patients (Wilson, 2000).

However, since the middle of the 20th century thinking about knowledge has changed. Sociological studies of science and knowledge production and studies of language have argued that objectivist views of knowledge do not correspond with the practice of knowledge production and the meaning of knowledge in daily life (Barnes and Bloor, 1982; Latour and Woolgar, 1979; Wittgenstein, 1953). Even within science pure objectivity and neutrality are impossible. Just like non-scientists scientists live and work in social contexts and have cultural values and personal interests that influence experimentation and observation and, thus, processes of knowledge production. These insights led to the emergence of new, relativistic perspectives on knowledge and truth. As a result, many contemporary scholars refrain from disqualifying patients' experiential knowledge, since they deny the existence of one absolute truth,

emphasizing instead the socially constructed or contextual character of all knowledge, scientific knowledge included.

Within these more relativist perspectives on knowledge the question arises how to distinguish between 'non-sense claims' and 'knowledge'. A pragmatist approach offers a solution. Pragmatists reject philosophical concerns about 'how the world really is', while recommending the philosophical importance of what is profitable or useful (cf. Rorty, 1982). They argue that a knowledge claim is acceptable if, and only if, this acceptation is *useful* to us. Starting from a pragmatist perspective, the experiential knowledge of patients, thus, can be considered valid within a certain context, if it proves to be useful within that context[34]. Following this line of argumentation, the validity of experiential knowledge of patients is generally acknowledged in different contexts, such as the development of individual coping strategies, the mutual understanding and mental support of fellow sufferers, and individual health care decision-making. But could the experiential knowledge of patients be considered useful within the context of biomedical research as well?

3.2 A pragmatic reflection

The investigation of the usefulness or practical value of patients' experiential knowledge with respect to its (potential) beneficial contribution to the relevance or content of biomedical research requires the study of concrete examples of individual biomedical research processes that in some way have been changed by the inclusion of this knowledge. If we can identify at least a few successful examples, we have an indication of the potential value of patients' experiential knowledge for biomedical research.

3.2.1 Methodology

The first stage of this study began with a search for cases of patient participation in biomedical research. Initially data were gathered by conducting an extensive literature and Internet search. However, since this

[34] This kind of argument is applicable to all three types of knowledge, whether from patients or scientists.

topic of study is rather poorly documented, most data had to be collected through personal communication. To this end 42 exploratory or explanatory, semi-structured interviews were held in the Netherlands and the United Kingdom. Interviewees comprised sixteen patients and patient representatives from different patients' organizations, seven medical or biomedical scientists, and nineteen other professionals from intermediate organizations (such as research councils, societal research institutes, research funding agencies, and science shops). Through these interviews we obtained a good representation of the different stakeholders within the field of patient participation in research. Interviewees were purposefully selected or selected by using the snowball method, while searching for people with an interest in, or experience with, patient participation in research. In the interviews we asked respondents to name examples of patient participation in biomedical research processes, and discussed the general concept of experiential knowledge of patients and its potential value for biomedical research. By this method 23 cases of patient participation in biomedical research were identified.[35]

Since patient participation does not necessarily imply the use of patients' experiential knowledge, these 23 cases were further investigated during the second stage of our study. We measured the concrete input of patients' experiential knowledge and the extent to which this knowledge had influenced biomedical research processes, for example by introducing new research topics or changing research programmes. Besides additional literature research, 20 additional interviews were conducted with previous (3) as well as new (17) interviewees (one biomedical scientist, eight representatives from patients' organizations and eleven representatives from intermediate organizations). New interviewees were selected on the basis of their personal involvement in one of the cases under investigation. In this process, many cases dropped out because the concrete input of patients was hard to recover. From the 23 cases, we identified nine that we considered clear examples of the actual use of patients' experiential knowledge in biomedical research processes. Although a more extensive analysis of all cases of patient participation identified would probably have resulted in a higher number of concrete examples, a small number of examples is considered sufficient for the purpose of making an argument for the practical value of patients' experiential knowledge for biomedical research.

[35] The interviews conducted as well as the cases identified partly overlap with the interviews and findings described in chapter 2.

3.2.2 Results

Table 3.1 shows an overview of the 23 identified cases of biomedical research processes in which patients have played a role.

Table 3.1 Cases of patient participation in biomedical rese urch

1.	The Advisory Council on Health Research (RGO – 'Raad voor Gezondheic onderzoek') in the Netherlands, which advises the government on national research p ogrammes and priorities, has one patient member. Patients are also involved in tempora programming and prioritization subcommittees.
2.	The United Patients' Organizations of the Chronically Ill (WOCZ - 'Werkverband Organisaties Chronisch Zieken') in the Netherlands introduced research on o-morbidity as a research priority within an integral programme on chronic illnesses of ie Netherlands Organization for Scientific Research (NWO-MW).
3.	After years of lobbying, the Dutch Alliance of Parents' and Patients' Orgar zations (VSOP – 'Vereniging Samenwerkende Ouder- en Patiëntenorganisaties') is increasi gly involved in national and international policy making on genetic research. It also has e ablished seven academic chairs on topics such as clinical genetics and rare diseases.
4.	During the past ten years the Dutch Society of Haemophilia Patic its (NVHP – 'Nederlandse Vereniging van Hemofilie Patiënten'), alongside other patier organizations, has successfully lobbied for more governmental support for research on ger tic therapy.
5.	The Canadian Breast Cancer Research Initiative (CBCRI) is the primary fir ncier of breast cancer research in Canada. Since the beginning, breast cancer survivor have been an integral part of the Initiative, helping to set research priorities alongside esearchers and clinicians.
6.	In the Netherlands, two patients participate in the Steering Group on Orphan Drugs ('Stuurgroep Weesgeneesmiddelen'), an organization that stimulates and fa litates research on, and development of, orphan drugs.
7.	Four representatives of the Dutch Neuromuscular Diseases Associ tion (VSN – 'Vereniging Spierziekten Nederland') are members of the board of the Du ch Foundation for Neuromuscular Research (SONMZ – 'Stichting Onderzoek Neuromus ulaire Ziekten') that aims to stimulate and communicate scientific research on causes of, ar l therapies for, neuromuscular diseases.
8.	The Medical Research Council in the UK has its own Consumer Liais on Group that advises on research priorities and programmes.
9.	Several patient members participate in the societal advisory council as w ll as in several programme committees of the Netherlands Organization for Health Research and Development (ZonMw).
10.	Within the programme 'Quality research on dementia' of the Alzheimer' Society in the UK, patients play a central role in research priority setting and in the appr sal of research projects.
11.	Through intensive interaction with scientists, patients from the French Mus ular Dystrophy Association (AFM – 'Association Française contre les Myopathies') are acti ely involved in

the agenda setting of biomedical research on neuromuscular diseases in Fra ce.

12. The European Platform for Patients' Organizations, Scientists & Clinicia s and Industry (EPPOSI) comprises several patient board members. Among others it aims to find ways to promote funding and facilitate the development and availability of inn vative medical solutions to all individuals in need.

13. The National Breast Cancer Coalition in the USA has brought about the a ceptance of the idea that breast cancer survivors must be involved in decision-making on research policy and research funding.

14. In the USA, the Council of Public Representatives (COPR) has been establi hed in order to enlarge the influence of the public on the National Institutes of Health (NIH). COPR members review and advise on NIH priorities and mechanisms for publi input to NIH decisions.

15. The German Retinitis Pigmentosa patient group Pro Retina has bee successful in influencing scientific research on Retinitis Pigmentosa in Germany. It h s been able to intervene in the scientific community, lobby for public funding, for ulate research priorities, and fund innovative research projects.

16. In the Netherlands, two patients participate in the scientific advisory council of the National Rheumatism Foundation (NRF – 'Nationaal Reumafonds') that ap raises research proposals on both scientific quality and societal relevance and advises th board of the Foundation about sponsoring.

17. In Australia, patients participate in several research committees, supported and stimulated by the Consumer's Health Forum.

18. Complaints and questions from Dutch patients with neuromuscular dis ses about the severe fatigue they suffered led to the launch of a new research project n Nijmegen on central and peripheral aspects of muscular fatigue.

19. The Dutch Kidney Patients' organization (NVN – 'Nierpatiënten Verenig ng Nederland') stimulated the launch of a research project on causes of, and therapies fo , 'restless legs', which is funded by the Dutch Kidney Foundation (NSN – 'Nierstichting N derland').

20. The Dutch Addison and Cushing Society (NVACP – 'Nederlandse V ereniging voor Addison en Cushing Patiënten') approached a science shop in Utrecht with the request for a study on drug administration methods in order to search for improve medication of Addison's disease. This study eventually led to the establishment of a resea ch project on a new delayed release tablet.

21. The mother of a young woman with adenocarcinoma of the vagina sug gested that her daughter's disease might have been caused by the drug diethylstilbestrol. This hypothesis led to new research on, and the eventually proof of, the teratogenicity of DE S.

22. The Hyperactive Children's Support Group is a patients' organization that formulated the hypothesis that hyperactivity of children can be caused by a deficiency o essential fatty acids. The hypothesis was picked up by the research world, resulting in m re research on the topic.

23. Female patients with Crohn's disease experienced that the metronidazole t ey received for curing a vaginal infection had a positive effect on their bowel disease as w ell. This led to additional research resulting in a new application of the drug.

We found that patient participation in biomedical research was mainly restricted to decision-making at a national or institutional level in stages such as general research policy, research programming, and research prioritization (cases 1-17). Within individual research projects, patients were occasionally involved in the identification of research topics or questions (cases 18-23). No cases were found in which patients participated in 'core' stages of biomedical research, such as research design, execution, and interpretation of results.[36]

Within the cases identified, we distinguished three different types of patient input:

- *demands* concerning (new) research priorities (cases 1-15) or research topics (cases 18-20),
- *ideas* on aetiological or therapeutic aspects of diseases or symptoms (cases 21-23), and
- *judgements* about the relevance of specific research priorities or projects (9-17).

Since they are based on personal experience of disease, symptoms, therapy, etc., we regard these demands, ideas, and judgements as different manifestations of patients' experiential knowledge. They build on both implicit and explicit forms of experiential knowledge, thus making this knowledge visible and applicable within biomedical research processes.

However, as mentioned before, the concrete input of patients (concrete demands, ideas, or judgements) and their influence was still indiscernible within the majority of the cases found. After detailed analysis of the cases, we identified nine examples in which the actual contribution of patients' knowledge to, and its impact on, biomedical research processes could be clearly traced. These examples are briefly described in table 3.2, listed according to the type of input provided.

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[36] By comparison, patients do increasingly participate in 'core' stages of clinical research processes, such as trials that involve the testing of new biomedical technologies in clinical practice. Within clinical trials, patients are progressively more involved in decision-making on research design and evaluation, especially concerning ethical aspects, patient information provision, and informed consent procedures (Hanley et al., 2001; Koops and Lindley, 2002; Oliver and Buchanan, 1997; Thornton, 1998). One of the reasons is that clinical research heavily depends on the recruitment of patients as research objects. Apart from increasing the quality and relevance of the research, patient participation in research design and evaluation is expected to increase the willingness of patients to participate as trial objects (Hanley et al., 2001).

The examples mentioned in table 3.2 show that the manifestation of patients' experiential knowledge is able to influence biomedical research at different stages of the research process. Patients' demands (needs or concrete questions) for research led to the formulation of additional research priorities within national and international research programmes (1-3) or new research topics or questions to be investigated and, thus, to the launching of new research projects (4-6). Patients' ideas on aetiological or therapeutic aspects were translated into new biomedical hypotheses or research questions (7-9).

Table 3.2 Examples of the use of patients' experiential knowledge in biomedical research

Demands

1. The United Patients' Organizations of the Chronically Ill (WOCZ) in the Netherlands was asked by the Netherlands Organization for Scientific Research (NWO) to formulate prioritization criteria for part of an integral programme for research into chronic illnesses. The WOCZ put forward the theme of co-morbidity as a research priority, a badly researched phenomenon many chronically ill patients have to deal with. Subsequently, NWO included this priority in the research programme.

2. Patients of the Alzheimer's Society in the UK annually decide on research priorities for the programme 'Quality research on dementia'. Priorities they identified for the research programme 2002 were amongst others epidemiological research on risk factors for dementia, vaccine research, and research on alternative and complementary therapy (Alzheimer's Society, 2002). Research applicants are forced to stick to these priorities since they have to state on their application form which priority area their proposal fits into.

3. In the Netherlands, a national research programme on pain ('Stimulation of pain research'), programmed by the Advisory Council on Health Research (RGO) in 1991, has been influenced by patients. The patient community felt that the study of the 'careers' of patients with chronic pain within the care system should be one of the main priorities within the programme. The patient member of the programming committee was able to push forward this priority. Even in the current, third stage of the programme, research on 'pain careers' is still a priority (Raad voor Gezondheidsonderzoek, 2001).

4. Several patients with neuromuscular diseases approached their medical specialist in Nijmegen in the Netherlands with complaints and questions about the severe fatigue they suffered from. They experienced this fatigue as very disabling and different from 'normal' fatigue, and asked for more research on this symptom. This made the specialist launch a new research project on central and peripheral aspects of muscular fatigue which is now performed by the Department of Neurology, University Hospital Nijmegen.

5. Many patients with kidney disorders suffer from, and complain about, so called 'restless legs', a symptom that causes serious insomnia. A biomedical researcher published an article on the subject in the bimonthly magazine of the Dutch Kidney Patients' organization (NVN). A request of the NVN for reactions to this article resulted in a stream of patient reports. Subsequently, the NVN made a strong plea for more research on this symptom

(Want, 1995). In response, the researcher submitted a research proposal on he topic to the Dutch Kidney Foundation (NSN). As a result, the Centre for Sleep and Wa e Disorders in The Hague is conducting a research project on both the prevalence of, and he therapy for, restless legs among kidney patients.

6. Since patients with Addison's disease need to take substitutive hydrocorti one every few hours in order to adequately suppress the different symptoms, many pa ents complain about the need to get up at night to take their medicine. Therefore the Dut n Addison and Cushing Society (NVACP) approached the Science Shop for Medicines in U recht with the request for a study on drug administration methods in order to search f : an improved medication of Addison's disease. The Science Shop commissioned an inve itory literature study on the subject. Subsequently, a professor of pharmaceutics took up the topic and launched a research and development project on a new delayed release ydrocortisone tablet in collaboration with a small Danish pharmaceutical company.

Ideas

7. The mother of a young woman with adenocarcinoma of the vagina suggest d that the fact that she had taken the drug diethylstilbestrol during her pregnancy might b related to the disease of her daughter. The oncologist who treated the young ill wo man took the suggestion seriously and started a systematic investigation on the relatio n between the exposure to DES in foetal life and the subsequent development of vaginal a lenocarcinoma (Ulfelder, 1980). Nowadays, the teratogenicity of DES is well known world- vide.

8. Women with Crohn's disease experienced that the metronidazole they recei ed for curing a vaginal infection had a positive effect on their bowel disease as well. The reported their experiences to their medical doctors, who took their finding seriously. his led to the execution of additional research on the drug. Nowadays, metronidazole is r gularly used in the treatment of inflammatory bowel diseases as well.

9. After an elaborate study on the characteristics of their children and the r families, the patients' organization Hyperactive Children's Support Group formulated he hypothesis that the hyperactivity of many of these children was caused by a deficie cy of essential fatty acids. The hypothesis entered the research world in a scientific artic published by the Support Group itself (Colquhoun and Bunday, 1981). After additio nal biomedical research, the hypothesis of a relation between a deficiency of essential atty acids and hyperactivity received considerable support (Stevens et al., 1995).

Although nine out of the 23 identified cases concerned judgements, we did not find any concrete examples of patients' judgements influencing biomedical research processes. Patients' judgements on research priorities and research projects usually play a role within the context of a committee. However, because of the complexity and opacity of decision-making processes in most committees, it is very difficult to determine whether and to what extent the input of patients has influenced decision-making (see also Oliver et al., 2004: 18). Final decisions are the result of many discussions and negotiations, in which the specific contribution of patients is hardly recoverable and usually can only be guessed at. A further complication in studying this kind of participation

is the privacy agreement implicit in the rules of most committees. Members usually do not want to give detailed inside information on decision-making processes.

3.3 Concluding remarks

In this chapter it has been argued that patients acquire specific knowledge based on their recurrent experience with their body, their illness, and the health care system. It is not easy to make a conclusive judgement on whether this knowledge can be considered valid, since this judgement depends on the paradigm adhered to. Following a pragmatist approach, we therefore decided to estimate the validity of patients' experiential knowledge in terms of its practical value and conducted a study for the identification and analysis of concrete cases in which this knowledge was applied in biomedical research processes, as described in the previous section. This study indeed suggests that this knowledge has potential value for biomedical research. In the identified examples patients' experiential knowledge had been translated into explicit demands or ideas that formed a direct input into biomedical research processes. Whereas the demands of patients may be considered as emerging from experienced problems (for example co-morbidity and chronic symptoms) or wished solutions (for example alternative therapies, vaccines, convenient drug doses) identified by patients, in these examples patients' ideas can be considered as directly pertaining to biomedical hypotheses or research questions. Therefore the findings refute the notion that contributing to biomedical research requires highly specialized knowledge. Since it is chiefly the biomedical researchers themselves who in these examples decided to include the manifestations of patients' experiential knowledge in their research processes, they can be considered to have (implicitly) acknowledged the relevance (or validity) of this type of knowledge.

In accordance with the findings in chapter 2, the findings in this chapter further indicate that the knowledge of patients, in spite of its potential, is only rarely included in biomedical research processes and often in an *ad hoc* fashion. In many of the identified cases, patients did not participate in research processes deliberately. Doctors in attendance (e.g. the neurologist working on neuromuscular fatigue, the oncologist working on DES, and the doctors consulted by women with Crohn's disease), patients' organizations (e.g. the Dutch Kidney Patients' organization, the

United Patients' Organization of the Chronically Ill and the Hyperactive Children's Support Group), disease specific research foundations (e.g. the Dutch Kidney Foundation and the Alzheimer's Society), or science shops (e.g. the Science Shop for Medicines Utrecht) acted as intermediaries between individual patients and the research world.[37] They (implicitly) made a distinction between useful and less useful experiential knowledge of patients[38] and often pursued a strategy of inter-subjectivity by gathering and selecting shared experiences and knowledge of a larger number of patients. In cases where no visible intermediary played a role (e.g. the example concerning the programming committee on pain research), involved patients were often active members of patients' organizations, who internalized and disseminated shared demands, ideas, and judgements themselves.

At the moment, only patients' judgements are occasionally involved in a more structural way when patients participate in specific committees, boards, or councils. But this form of participation does not ensure the actual use of these judgements. The eventual use of patients' experiential knowledge is influenced by a variety of factors, including the proportion of patient members to professional members, patients' empowerment, professionals' susceptibility to patient views, the actual locus of decision-making, etc. [39]

Various interviewees mentioned that successful patient participation in research committees requires that the patient has acquired a degree of proto-professionalism either by self-education or by training, since only such patients are generally regarded as appropriate discussants in professional surroundings. However, as has been stated before, proto-professionalism may lead to non-representation of the patient community and to the loss of 'pure' experiential knowledge (Mullen, 2000). Several interviewees stressed that in order to reduce the risk of losing specific patient perspectives, participating proto-professionalized patient representatives should stay in close contact with the patient population

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[37] They thus follow the 'second' strategy of patient participation as identified in chapter 2 (section 2.2).

[38] Both patients and professionals interviewed in our study stressed that ideas and questions from naïve – non proto-professionalized – patients are rarely useful for biomedical research.

[39] See also the discussion on partnership versus placation in relation to the 'third' strategy of patient participation represented in sections 2.2 and 2.3.

they represent, verifying the mutuality of demands, ideas and judgements regularly.

Due to lack of experience, it is indeed not yet known whether a more structural way of patient participation in biomedical research processes can be considered 'efficient'. Several interviewees thought that the knowledge of patients might be useful in some cases, but that it is hardly worth the trouble; it would take much time and effort to involve patients structurally while effecting only occasional or marginal improvements. There is thus a clear need for practical proof of principle. But it is here that we face an obstacle. Hardly any strategies are currently available to facilitate a structural and effective participation of patients in biomedical research processes. Many questions were raised by various respondents, such as how can we distinguish between sense and non-sense information from patients, what type of patients need to be involved, how can learning processes between researchers and patients be enhanced. Some of these questions might be answered by investigating the specific skills and activities of intermediaries that have successfully bridged the gap between individual patients and the biomedical research world more closely. A closer study of the working practices of committees which involve patients in decision-making might also provide some new insights. For this purpose, committees should provide more insight into their procedures and practices by for example reporting reflections on their working practices.

Another and possibly more profitable way of investigating patients' possible contribution more systematically is by experimenting with participation exercises building on experiences within other scientific fields. In the remaining part of this book we will take up this challenge. As a start, the next chapter will elaborate on a possible methodology for effective patient participation in decision-making on biomedical research.

4

A TRANSDISCIPLINARY STRATEGY[40]

Participation of patients in decision-making on biomedical research has been rare, and integration of patients' experiential knowledge in these decision-making processes – in the few cases it takes place – occurs implicitly and on an ad hoc basis. In order to optimize the use of patients' experiential knowledge in biomedical research, a systematic approach is required that comprises both consultation and collaboration steps. Since such an approach should systematically, explicitly, and deliberately integrate knowledge from different scientific and non-scientific sources, it can be called transdisciplinary. This chapter elaborates on the concept of transdisciplinarity and on the design of a possible transdisciplinary strategy that could be used to realize effective patient participation in decision-making on biomedical research. For this purpose a possible procedure is proposed and necessary conditions and skills are identified. In addition, the feasibility of its implementation within the biomedical sector is discussed.

One of the main findings of this study thus far is that patient participation in decision-making on biomedical research is far from being common practice. Patients and their experiential knowledge are seldom involved in decision-making on biomedical research and if they are, it is in a sub-optimal way and on an ad hoc basic. As a result, new and relevant ideas or directions for biomedical research might be missed.

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[40] The text of this chapter is partly based on: Flinterman, J.F., Teclemariam-Mesbah, R., Broerse, J.E.W., and Bunders, J.F.G. (2001). Transdisciplinarity: The new challenge for biomedical research. *Bulletin of Science, Technology & Society* 21(4): 253-266.

In chapter 2 we argued that a more structural and effective participation of patients in decision-making on biomedical research agendas would imply the inclusion of patients in the biomedical research decision-making network, which requires considerable changes in current ways of thinking and acting. This change of the current decision-making network and its regimes can be considered a transition. We subsequently argued that such a transition could be induced, among others, in a bottom-up way by the execution of successful innovation experiments. The central question in this chapter is how such experiments could be designed. Therefore this chapter focuses on the development of a participation strategy[41], based on previous results, on literature research, and on earlier experiences of our Institute[42] with the development and implementation of interactive strategies.

As has been specified in section 2.3, we consider patient participation in decision-making effective if it enhances both the legitimacy and rationality of decision-making processes and the quality of decision-making outcomes. This implies the need for direct and early involvement of patients in fair decision-making processes and the actual integration of their knowledge in decision-making outcomes. As has been shown in chapter 2, current strategies for patient participation in decision-making on research agendas usually consist of either *consultation* or *collaboration* methods (see also Boote et al., 2002; Hanley et al., 2000; Oliver et al., 2004; Telford et al., 2002; Williamson, 2001). Consultation methods include interviews, questionnaires, focus groups, and citizens' juries, all aiming to identify patients' perspectives, needs, or priorities. Collaboration initiatives usually comprise the membership of one or more patient(s) in decision-making structures.[43] However, both strategies often fall short of realizing effective participation according to its definition mentioned above. Initiatives that only entail the consultation of patients tend to fail in ensuring patients' influence on research agendas since decisions concerning the use of patients' inputs are entirely in the hands of

[41] We use the term 'strategy' in order to express the societal relevance of the patient participation accomplished. However, from a pure scientific perspective, the strategy formulated in this chapter can be considered a methodology for effective stakeholder participation in general.

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[43] Collaboration thus can refer to both placation and partnership in Arnstein's participation ladder, depending on the degree of power sharing between patients and experts.

professionals (also mentioned by Abelson et al., 2003; Oliver et al., 2004). Initiatives that involve collaboration often fall short of procedural fairness and thus of legitimacy of the process. In addition, they neither ensure patients' influence – at least in the way collaboration exercises are currently structured. The majority of patients struggle to hold their own when facing a team of professionals; they easily become overruled by professionals causing the collaboration to degenerate into tokenism (see also Oliver et al., 2004: 88). Only considerably proto-professionalized and empowered patients are (or feel) treated on equal terms by professionals. However, since most reports on collaboration exercises do not distinguish between priorities set by patients and those set by professionals, the assessment of the actual influence of patients on decision-making outcomes is hampered (see also Oliver et al., 2004: 88).

We argue that a strategy for effective patient participation in decision-making on biomedical research should involve at least *both* a *consultation* and a *collaboration* phase. In a consultation phase patients' perspectives – consisting of demands, ideas, or judgements – as well as the perspectives of other stakeholders are made explicit. In a subsequent carefully-guided collaboration phase the deliberate integration of these perspectives, and thus of patients' experiential knowledge, in research agendas can be realized. In addition, in order to optimize this use of patients' experiential knowledge, processes of knowledge integration should be transparent and explicit, so that they can be studied, influenced, and optimized deliberately. A *transdisciplinary* approach, which by definition focuses on the integration of scientific with non-scientific knowledge, may offer a way to optimize knowledge integration into decision-making processes and thus to increase the effectiveness of patient participation.

This chapter elaborates on a transdisciplinary patient participation strategy that includes both consultation and collaboration steps. We start with discussing the concept of transdisciplinarity in the next section. Subsequently, we propose a procedure and identify conditions that could be part of a successful transdisciplinary strategy. Finally, we discuss the feasibility of the implementation of such a strategy within the context of biomedical research. We end this chapter with the announcement of a social experiment that aims to apply and evaluate the transdisciplinary strategy within the context of patient participation in research agenda setting.

4.1 The concept of transdisciplinarity

Transdisciplinarity can be compared with concepts of monodisciplinarity, multidisciplinarity, and interdisciplinarity. These concepts are mainly used within the framework of scientific research and can be considered as referring to different degrees of interaction and integration between different knowledge domains.

Monodisciplinary research is the most common form of scientific research. It is restricted to one research discipline, to one branch or specialization within a research field. People working within one discipline study the same research objects, share the same paradigm, use common methodologies, and speak the same 'language' (Aram, 2004; Judge, 1991; Klein, 1996; Salter and Hearn, 1996). When a variety of disciplines collaborate in one research programme without integration of concepts, epistemologies, or methodologies, we speak of multidisciplinarity. In multidisciplinary research, the degree of integration between disciplines is restricted to the linking of research results. Interdisciplinarity is also a collaboration of several disciplines, but in this case concepts, methodologies, or epistemologies are explicitly exchanged and integrated, resulting in a mutual enrichment (Gibbons et al., 1994; Jantsch, 1972; Klein, 1996; Salter and Hearn, 1996). Different degrees of interdisciplinarity can be distinguished depending on the degree of exchange and integration and the differences in paradigms between the disciplines involved.

Transdisciplinarity is a recent trend within interdisciplinarity in which boundaries between and beyond disciplines are transcended. It has been defined as "a new form of learning and problem solving involving cooperation among different parts of society and academia in order to meet complex challenges of society" (Klein, 2001: 7). Its goal is to get a better understanding of the present world as a whole by integrating various scientific and non-scientific perspectives of reality in search of a more holistic or 'socially robust' knowledge. This knowledge is characterized by its stronger orientation towards the context of application, the inclusion of stakeholder perspectives, and its problem-solving capability (Gibbons et al., 1994; Klein et al., 2001; Nowotny, 2003; Nowotny et al., 2001; Rapport, 1997; Scholz et al., 2000)[44]. Transdisciplinary approaches can be

[44] Another frequently used term to indicate this type of transdisciplinary knowledge, introduced by Gibbons et al. (1994), is 'Mode-2' knowledge. Mode-2 knowledge can be opposed to the traditional, academic, mono-disciplinary mode-1 knowledge.

practised in academic (research) contexts as well as in societal problem-solving or decision-making contexts.

One of the main challenges in transdisciplinary approaches is how non-scientific knowledge can be validated and integrated with scientific knowledge. Therefore, we elaborate on the concept of knowledge integration below.

Knowledge integration

In the previous chapter, we have argued that both scientific and non-scientific knowledge consist of three types of knowledge – propositional knowledge, procedural knowledge, and knowledge of acquaintance – that can be considered as reflecting different origins and contents, thus referring to a substantive dimension of knowledge. These three types of knowledge can all be involved in transdisciplinary knowledge production, problem solving, or decision-making.

Within the framework of understanding knowledge integration another helpful dimension of knowledge is explicit – implicit (or 'tacit', see Polanyi, 1966), which refers to its form of expression. Explicit knowledge is 'codified' knowledge that can be easily transmitted in formal or systematic language. It has also been described as the 'information' component of knowledge (Weggeman, 1997). Implicit knowledge comprises personal and context-specific knowledge that is hard to formalize and communicate. It can be considered as comprising experiences, skills, or attitudes, (Nonaka and Takeuchi, 1995; Weggeman, 1997). Propositional knowledge is explicit, while knowledge of acquaintance is mainly implicit. Procedural knowledge can be implicit or explicit, depending on the degree of conscious reflection and expression.

The integration of scientific and non-scientific knowledge thus includes the integration of explicit forms of knowledge as well as the integration of more implicit forms of knowledge. Integration of explicit knowledge could involve processes of translating and transferring of information; analyzing, structuring, and clustering of information; searching for overlapping, connecting, and supplementing elements; making common factors explicit; negotiating; etc. It is what Nonaka and Takeuchi have termed 'combination' (1995: 67-69). In principle, explicit knowledge from different stakeholders, after it has been gathered, could be integrated in final decisions or solutions without involving those stakeholders themselves in the actual integration process. Methods for knowledge integration mentioned in this context include methods that are

used as well within interdisciplinary research, such as methods based on systems thinking and (computer) modelling (e.g. Scholz et al., 2000).

Since implicit forms of knowledge cannot be transferred or exchanged between stakeholders in an oral or written way, they have to be integrated in decisions or solutions by direct, personal involvement of these stakeholders in decision-making or problem-solving processes ('socialization' in terms of Nonaka and Takeuchi, 1995: 62-64). Only the knowers themselves are capable of using their implicit knowledge and can translate this knowledge into concrete demands, ideas, judgments, etc. that influence the outcomes. Within this framework, Regeer and Bunders (2003) describe knowledge integration as a process of knowledge creation that can only take place within 'communities of practice'. Methods that could be used in order to facilitate or stimulate the inclusion of implicit forms of knowledge within transdisciplinary knowledge integration include all kinds of participatory and deliberative methods, such as dialogues, interactive workshops, consensus conferences, focus groups, Delphi techniques, etc. (e.g. Klein, 2001; Zweekhorst et al., 2001). In order to ensure the achievement of effective knowledge integration, communication and interaction between the different actors involved within these methods should be carefully guided.

An important issue in transdisciplinary knowledge integration concerns the validation of the knowledge that is to be integrated as well as the validation of the outcomes of the knowledge integration process. Discussions about the validity of knowledge usually take place within the domain of epistemology, which traditionally focuses on explicit, propositional knowledge only. Validation of explicit knowledge claims has much to do with their verification by testing on the basis of certain criteria. Different types of knowledge ask for different validation criteria (Schipper, 1999). For example, traditional scientific propositional knowledge, as found in the natural sciences, generally strives to be objective and universally valid. For this knowledge, important validation criteria will be (logical) consistency and empirical adequacy (De Wilde, 1989). On the contrary, non-scientific propositional knowledge (and in particular experiential knowledge) is more subjective and contextual. Instead of universal validity, this knowledge seeks for validity in terms of (practical) value. It thus needs other validation criteria, such as applicability and contextual adequacy. Validation of this type of knowledge often occurs implicitly in society, for example when many people subscribe to the adequacy and appropriateness of an explanation, resulting in a degree of

inter-subjectivity, or when an explanation proves to be applicable in practice.

Contrary to explicit knowledge, implicit forms of knowledge are more difficult to investigate and validate. Since they are hard to communicate, they cannot be simply subjected to validation criteria. Instead, manifestations of this knowledge – such as demands, judgements, or ideas – could be validated, for example on the basis of their applicability or contextual adequacy. In this way implicit knowledge can be indirectly validated on its practical value (see for example section 3.3).

Transdisciplinary knowledge, as the result of knowledge integration of different types of knowledge, needs to be evaluated on the basis of different validation criteria, in order to ensure its validity. Meeting these criteria might give rise to interpretations or innovations that are not only scientifically adequate but also appropriate to the specific context of application and to the needs and demands of society. It implies a continuous dialogue between the different parties involved, with feedback loops for the crosschecking of previous assumptions, insights, and demands. In table 4.1, the main characteristics of a transdisciplinary strategy, as described above, are summarized.

Table 4.1 Main characteristics of a transdisciplinary strat gy

- holistic and integral approach
- acknowledgement of complex context and set of actors (scientific and soci al)
- orientation on societal perspectives and problem-solving capability in he context of application
- integration of explicit and implicit knowledge from various scientific an non-scientific sources
- direct and personal involvement of relevant stakeholders in participatory p ocesses
- evaluation on the basis of different validation criteria, such as consis ncy, empirical adequacy, applicability, and contextual adequacy
- dynamic, complex process with iterative feedback loops

4.2 A transdisciplinary strategy for patient participation

A successful transdisciplinary participation strategy covers different actions and conditions. First, a systematic process design needs to channel the

process. In addition, to ensure the effectiveness of such a procedure, the social setting, which determines the interactions between participants, has to meet certain conditions. Finally, members of the process management team who initiate and guide the transdisciplinary process have to possess or acquire some specific qualities and skills.

Until now, general systematic guidelines for a transdisciplinary strategy have not yet been developed. However, within several scientific and societal contexts interactive or participatory strategies have been developed for involving end-users and other societal actors in decision-making processes (e.g. Broerse and Bunders, 1999; 2000; Driessen et al., 2001; Grin et al., 1997; Johnson et al., 2003; Kasemir, Jäger et al., 2003; Reuzel, 2004; Rip et al., 1995; Tress and Tress, 2003). Since these strategies focus on the interaction between both scientific (or professional) and non-scientific (or lay) actors, as well as on the construction of an integral knowledge, they offer many procedural and conditional clues for a transdisciplinary strategy. In this section, we elaborate on the different components of a transdisciplinary strategy for the implementation of effective patient participation in decision-making on biomedical research.

4.2.1 Process design

Below, we propose a tentative transdisciplinary patient participation strategy based on the so-called Interactive Learning and Action (ILA) approach[45], an interactive strategy that has been developed by members of the Athena Institute[46] for the gearing of agricultural innovation projects towards the needs and interests of small-scale farmers in developing countries (see box 4.1) (Broerse, 1998; Broerse and Bunders, 1999; 2000). Reasons for choosing this approach are (1) it is a rather well elaborated and tested strategy that has proven to be successful in involving lay people in decision-making processes concerning science and technology, (2) we want to learn about the broader applicability of this strategy, and (3) we want to build on the rich experiences of members of our Institute with this participation strategy.

The ILA methodology should not be regarded as a blueprint; it only provides guidelines and within the boundaries of the key characteristics it needs to be adapted and specified to the context of

[45] formerly called the 'Interactive Bottom-Up approach'

[46] Athena Institute for Research on Innovation and Communication in Health and Life Sciences, Faculty of Earth and Life Sciences, Vrije Universiteit Amsterdam

application. In this study the ILA has been adapted to the context of patient participation in decision-making on biomedical research agendas.

Box 4.1 The ILA approach

Key characteristics of the ILA approach are the enhancement of trust relationships, mutual learning and knowledge integration between relevant stakeholders involved in a carefully guided process that is facilitated by a research team. Specific attention is paid to stakeholder groups that have previously been neglected in decision-making. To guide the process, the ILA approach is roughly structured along four phases (Broerse and Bunders, 2000).

1. *Initiation and preparation* This phase consists of (a) the establishment, and if necessary training of, a research team that is to guide the overall project, (b) the gathering of preliminary, contextual information, and (c) the definition of objectives and roles. During this phase insight is obtained on the problem situation and the actor network, and the feasibility of the interactive exercise is assessed. At the end a 'go/no-go' decision is taken with respect to the project.
2. *Collection, exchange, and integration of information* In this phase the team identifies and analyses perspectives, needs, and interests of the different stakeholders and assesses the status of current research. Subsequently, knowledge, perspectives, and needs of the stakeholders are mutually exchanged and integrated. This phase results in a thorough understanding of the problem and solutions from the perspective of the different stakeholders and an increased mutual understanding on the part of the various actors involved in the process.
3. *Public priority setting and planning* The third phase allows all actors involved to review and reflect upon preliminary results from the previous phase, to identify priorities, and to establish a plan of action. Often this phase comprises an interactive priority setting and planning workshop.
4. *Project formulation and implementation* The plan of action which resulted from the previous phase forms the input to the fourth phase, in which specific programmes or projects are formulated and implemented.

Besides these general guidelines on how to structure activities, the ILA approach includes a rich tool kit of methods and techniques for knowledge generation and interaction from which the research team can draw as required. These methods and techniques range from literature study, various types of interviews and questionnaires, to a wide variety of group-based methods such as focus groups, and workshops designed for different purposes including brainstorming, dialogue, integration and consensus. Also various visualization, diagramming and prioritization techniques are included.

As has been argued above, in order to ensure the explicit integration of patients' perspectives and knowledge in decision-making, consultation and collaboration methods should be combined in a structured process. In the original ILA approach knowledge integration between stakeholders involved alternates with the gathering of knowledge, both taking place in the second phase. Therefore in our transdisciplinary strategy we have divided the second phase into a separate consultation and a collaboration phase.

A possibly appropriate design of a transdisciplinary strategy for implementing effective patient participation in decision-making on biomedical research thus could be as follows:

1. *Preparation and initiation phase*
   In the first phase the biomedical field of interest is defined and the different stakeholders involved in that field, such as scientists, health care professionals, patients or patients' organizations, and policy makers, are identified and contacted. Current patterns of thinking and decision-making are estimated and (possible) obstacles and opportunities for the implementation of patient participation are identified. For this purpose desk studies, literature surveys, and various exploratory and in-depth interviews can be conducted. Finally, a transdisciplinary team is established in order to facilitate and guide the next two phases of the transdisciplinary process.
2. *Consultation phase*
   When the societal context has been assessed, in the consultation phase information and knowledge is gathered about the perspectives and views of the different stakeholders – including both central actors of the decision-making network and patients. This can be done via a variety of consultation techniques, such as exploratory and in-depth interviews, brainstorming sessions, discussion meetings, focus groups, and questionnaires. In order to investigate the separate perspectives of stakeholders and their mutual differences and overlaps, different stakeholder groups are not mixed at this stage. The preliminary findings – be they expressed research needs, relevant research topics, proposed research questions, criteria for priority setting, dilemmas encountered, or suggestions for further action – are laid down in an intermediary document.

3. *Collaboration phase*
   In the third phase the different stakeholders are brought together in interactive workshop or dialogue settings to review and discuss these intermediary findings, to exchange, combine, and negotiate views and perspectives, and to seek consensus concerning research needs, priorities, topics, etc. These workshops or dialogues need to be characterized by close and carefully guided interactions between the participants in order to facilitate mutual feedback, mutual learning and the development of shared constructions. This phase should ensure the explicit integration of patients' perspectives and priorities in the outcomes.
4. *Prioritization phase*
   Subsequently, in the prioritization phase participants are allowed to reflect on the results of the collaboration phase and to identify final priorities. In order to prevent that professionals or experts unintentionally overrule patients, this prioritization should be preferably done on an individual basis. Since the precise outcome of a priority setting strongly depends on which actors are involved, one should see that the participants in this phase adequately represent the different stakeholders involved, both in number and in characteristics.
5. *Specification phase*
   The fifth phase comprises the translation and specification of priorities identified into a concrete plan of action, including e.g. research programmes, projects, or policy actions. Although this phase is often in hands of the 'assigning body' that has commissioned the patient participation initiative, the involvement of stakeholders can ensure that the resulting plan of action reflect their priorities.
6. *Implementation phase*
   In the final phase, the plan of action established in the previous phase is implemented. This phase usually is in the hands of the 'assigning body' as well. Ideally also in this phase stakeholder groups are involved.

Each phase within such a transdisciplinary strategy consists of activities that can be undertaken several times, leading to an interactive, dynamic process. When 'all' information, knowledge, perspectives, ideas, demands, judgements, etc. are specified, analyzed, cross-checked, and integrated, the result is a shared construction of, and an integral vision of, the problem concerned or a possible course of action to be followed. The

procedure can be (successively) used to identify priorities and directions for research policy; to formulate research programmes; to identify, assess, and prioritize research topics; to formulate research questions and objectives; and to design research projects in a transdisciplinary manner. In table 4.2, the most important elements of the process design are listed.

Table 4.2 Process design of a transdisciplinary strateg

1.	*Pre ration and init tion phase*	-	definition of a field of interest
		-	identification and contacting of all relevant actor
		-	establishment of a process management team
2.	*Co ultation phase*	-	identification of stakeholders' views, perspectiv , needs, ideas, etc.
		-	literature research
		-	in-depth interviews, discussion meetings, focus g ups, etc.
3.	*Co boration phase*	-	development of shared constructions and an inte ral vision
		-	interactive workshops, dialogues, etc.
		-	repeated feedback on all kinds of results by all pa icipants
4.	*Pri itization phase*	-	individual or collaborative prioritization of result
5.	*Spe fication phase*	-	translation and specification of priorities identifie
		-	establishment of plan of action
6.	*Imp mentation phase*	-	implementation of plan of action

4.2.2 Social Setting

Central elements within transdisciplinary strategies are brainstorming sessions, discussion meetings, and interactive workshops that aim to enhance effective interaction between participants and integration of their knowledge. To make effective knowledge integration possible, however, the social setting of these meetings has to meet certain conditions. Within the context of the ILA approach, Broerse and Bunders (1999) have identified conditions for successful transdisciplinary social interactions. A first and indispensable condition is that the 'central' participants in the research process be committed to a shared vision with regard to the overall objective of the collaborative undertaking and on the importance of transdisciplinarity and knowledge integration in this process. Secondly, a kind of coalition building between the participants ensures optimal collaboration and the achievement of joint efforts and outcomes. The

atmosphere should be open and trustful and stimulate rational discourse, mutual learning, and feedback (in accordance with the theory of communicative action as described by Habermas, 1984; 1987). In addition, participants must have more or less equal access to information, support, funds, and other means. Central issues, such as overall research objectives and mutual expectations, have to be made explicit and formulated clearly for all participants. Finally, when established structures are to be changed during the process, the existence of some room for manoeuvring and negotiation is an additional condition.

Because most of these conditions usually will not be met in a real-world setting, a specific social setting conducive to the implementation of a transdisciplinary strategy often has to be created. For example, central participants can be selected based on whether they are open-minded towards other perspectives and willing to discuss their own. Although the deliberate selection of participants implies the initial establishment of a somewhat artificial situation, in many cases it is the only way to facilitate the development of a shared construction. Later in the process, a real-world setting can be pursued. In addition, the social setting needs to be carefully managed in order to facilitate mutual respect, openness, and learning. In table 4.3, abovementioned conditions for the social setting of a transdisciplinary strategy are summarized.

Table 4.3 Conditions for a transdisciplinary social setti g

- commitment to a shared vision
- coalition building
- equality in roles and means
- openness
- mutual respect
- mutual learning
- clarity in research objectives, strategies, expectations, and so on
- room for manoeuvring and negotiation

4.2.3 Team qualities and skills

To be capable of creating and facilitating the necessary social setting and applying the proposed procedural steps, process managers who guide a transdisciplinary strategy need to possess or acquire some specific qualities

and skills. First and foremost, they need to be able to transcend disciplinary boundaries and to respect and value non-scientific knowledge. Furthermore, they should possess a number of basic 'scientific' skills. Because in transdisciplinary strategies knowledge from various sources is collected, analyzed, and integrated, more is needed than simply the scientific skills used in traditional monodisciplinary research. Julie Thompson Klein (1996) formulated scientific skills for interdisciplinary and transdisciplinary research, including the gathering, translating, analyzing, weighting and valuing, structuring, and synthesizing of scientific and experiential information and knowledge. In addition, various communication and organizational skills are indispensable for conducting specific transdisciplinary activities, such as interviewing actors with different backgrounds, facilitating collaboration between these actors, and organizing workshops and discussion meetings.

To optimize the social interactions between the different participants, transdisciplinary process managers need to bridge communication and cultural gaps between the participants and play the role of intermediary. Bunders, Stolp, and Broerse (1991) identified several personal qualities and attitudes that are necessary for successfully mediating and guiding transdisciplinary processes, such as a broad interest, flexibility, creativity, openness, respectfulness towards people, perspectives, and cultures, and a tolerance to ambiguity. During the process, these intermediaries might slowly increase the awareness and receptiveness of the scientific world toward transdisciplinarity, thus acting as change agents as well. At the same time, they could play an important role in the

Table 4.4 Qualities and skills for transdisciplinary process managers

Qualities and Attitudes	*Skills*
- transcendence of discipline	- scientific skills for gathering,
- broad interest	translating,
- flexibility	analyzing,
- reflexivity	structuring,
- creativity	weighting and valuing, and
- openness	synthesizing knowledge and information
- respectfulness	- communication skills
- tolerance to ambiguity	- organizational skills
- willingness to act	

empowerment of marginal actors (Anbar, 1986; Broerse and Bunders, 1999; Schot and Rip, 1997). In table 4.4, the most important qualities and skills for transdisciplinary process managers are listed.

4.3 Implementation within the biomedical sector

Although various pleas are made for the inclusion of experiential knowledge of patients in decision-making on biomedical research, transdisciplinary decision-making on biomedical research is far from being common practice. Given the many conditions, qualities, and skills that are considered indispensable for a successful transdisciplinary strategy, as described in the previous section, one may even question the feasibility of a structural implementation of such a strategy. Is transdisciplinary decision-making on biomedical research simply a utopian dream, an ideal that cannot be realized? We have some indications that it might become a reality.

In different cases of patient participation in decision-making on biomedical research, as described earlier, several of the identified procedural elements of, and conditions for, transdisciplinarity have been present to some extent. For example, patients of both Pro Retina (box 1.1) and the French Muscular Dystrophy Association (box 1.2) have actively and successfully sought intensive interaction with scientists in discussion meetings or conferences that were characterized by mutual openness, respect, and learning. In other examples, intermediaries have successfully transcended disciplinary boundaries and have integrated patients' experiential knowledge in decision-making on scientific research topics or priorities. For this purpose they should have been open and respectful towards patients' perspectives and views. However, since in all those cases only few conditions for transdisciplinarity were fulfilled in an ad hoc and implicit manner, they do not provide conclusive proof that the implementation of transdisciplinary decision-making on biomedical research is feasible.

Another indication for the feasibility of successful implementation of our proposed transdisciplinary participation strategy can be derived from the different successful experiences with implementing the original ILA approach. In Ghana, Zimbabwe, and Bangladesh the ILA approach proved to be rather successful in involving end users and integrating their indigenous knowledge in agricultural priority setting and project design

(e.g. Broerse, 1998). Experiences in Bangladesh also showed the potential for institutionalization of this approach within established structures (Zweekhorst et al., 2003). Of course, further research still needs to investigate whether an ILA-based strategy is also applicable and effective in the context of biomedical research agenda setting.

4.4 A view ahead

The remaining part of this book describes a social experiment that aims to implement and evaluate the transdisciplinary strategy proposed above within the context of an interactive health research agenda-setting project, initiated and commissioned by the Netherlands Asthma Foundation (NAF) and co-financed by the Netherlands Organization for Health Research and Development (ZonMw). The project does not specifically focus on biomedical research but includes biomedical research in the agenda-setting process. Although this may somewhat complicate the further investigation of the feasibility of implementing transdisciplinary strategies within the biomedical sector, it does offer the additional possibility to investigate patients' interest in, and demand for, biomedical research with respect to other types of health research.

The context of asthma and COPD[47] research agenda setting initiated by the NAF seems to be quite suitable for implementation of a transdisciplinary approach. Firstly, both asthma and COPD are chronic illnesses, which enables patients to obtain experiential knowledge. Secondly, although it is generally acknowledged that both diseases are caused by multiple genetic and environmental factors, precise details on these factors are unknown. In addition, adequate therapies without negative side effects do not yet exist. Therefore there might be some room, or even a need, for new and alternative inputs from patients concerning biomedical research topics or questions. Thirdly, the Netherlands Asthma Foundation is both a research fund and a patients' organization – a rather unique combination in the Netherlands – which facilitates the gearing of research programmes and priorities towards patients' needs and demands, and makes the NAF a possible facilitative, intermediary platform for interaction between scientists and patients. The

[47] Chronic Obstructive Pulmonary Disease

next two chapters will describe and evaluate this social experiment in detail.

5

PATIENTS' RESEARCH PRIORITIES[48]

This chapter describes the consultation of Asthma and Chronic Obstructive Pulmonary Disease (COPD) patients on their priorities concerning health research. This consultation was an essential step within an interactive research agenda-setting project that was commissioned by the NAF and ZonMw. The overall project, which will be described and evaluated in the next chapter, aimed to apply and test the transdisciplinary participation strategy proposed in the previous chapter. An additional objective of the patient consultation step was to investigate the capability of patients to participate adequately in, and to contribute to, (biomedical) research agenda setting, which will validate the substantive argument in favour of this patient participation. For this purpose a consultation procedure was designed comprising seven focus groups, a feedback meeting, and a questionnaire. The focus groups and the feedback meeting aimed to explore the entire breadth of problems that patients experienced in relation to their diseases, while the questionnaire aimed to investigate patients' prioritization of possible research targets that focus on solving these problems. The consultation procedure successfully elicited patients' research priorities and their underlying arguments. Our results indicate that asthma and COPD patients are capable of research prioritization in a well-founded way and that they highly value biomedical research. Furthermore, since they prioritized some biomedical research topics that were not covered by current research programmes, we argue that patient participation will contribute to biomedical research agenda setting.

[48] The text of this chapter is based on Caron-Flinterman, J.F., Broerse, J.E.W., and Bunders, J.F.G. (2005). Patients' priorities concerning health research: The case of asthma and COPD research in the Netherlands. *Health Expectations* 8(3): 253-263.

In order to apply and test the transdisciplinary participation strategy, proposed in the previous chapter, a social experiment was conducted, comprising an *interactive research agenda-setting project*, commissioned by the Netherlands Asthma Foundation (NAF) and co-financed by the Netherlands Organization for Health Research and Development (ZonMw). This project is described and evaluated in detail in the next chapter.

This chapter only describes one, but essential, step within the project: the consultation of asthma/COPD patients on their health research priorities. Besides making patients' research priorities explicit, which is an important input in the agenda-setting process, the patient consultation step offers a way to investigate patients' capability of adequately identifying and prioritizing health research topics. Although the agenda-setting project does not specifically focus on biomedical research, biomedical research is part of it. Therefore, in an indirect way, we hope to be able to estimate patients' capability of contributing to biomedical research agenda setting as well. This investigation will address a difference of opinions concerning the desirability and feasibility of patient participation in health or biomedical research agenda setting.

As has been found in chapter 2, an important obstacle for effective patient participation in decision making on biomedical research is the opinion of many relevant actors (both researchers and patients) that patients should not contribute to decision-making on biomedical research agendas for various reasons. It was argued that patients:

- lack essential knowledge about research issues and procedures,
- do not speak nor understand scientific language,
- are unable to put their own questions and demands into a scientific context,
- have unrealistic expectations of scientific research,
- are strongly influenced by the media,
- are unable to abstract from their own individual situation,
- have difficulty to think in long-term targets, and/or
- are only interested in subjects concerning care or social issues.

As a result, many concluded that patient involvement in biomedical research agenda setting would be useless and that their involvement in overall health research agenda setting would result in an undervaluing and subordination of biomedical research.

Until now, available literature on the subject, such as systematically reviewed by Oliver et al. (2004), hardly provides evidence concerning the

tenability of above-mentioned presuppositions. Reports on patient consultations concerning research priorities often concern a restricted field of health research only, or do not distinguish patients' priorities from the priorities of professionals. But even if many of the above-mentioned presuppositions can be substantiated, it may be unfair to conclude that patient participation in overall health research agenda setting is useless and undesirable. Scientific knowledge, for example, may not always be a necessary prerequisite for useful participation. Indeed, patients may possess other types of knowledge of value and relevance to research agenda setting, as is argued by several scholars (Entwistle et al., 1998; Goodare and Lockwood, 1999; Popay and Williams, 1996). According to these scholars, a distortion of research priorities due to the inclusion of patients' knowledge can be positive, since this broadening of prioritization can counter potential biases of scientists and health care professionals.[49]

We used the consultation of asthma/COPD patients on their research priorities to investigate the tenability of the different presuppositions described above. For this purpose, we had to design an adequate methodology that consults patients about their research priorities in an explicit and transparent way. Such a methodology would provide greater insight into whether patients can prioritize health (and biomedical) research topics in a well-argued way and can make relevant contributions to current health or biomedical research agendas.

5.1 Methodology

From September 2003 to February 2004 we consulted asthma and COPD patients about their priorities on asthma and COPD research using a triangulated strategy. Focus groups were conducted to explore the entire breadth of, and to gain insight in, patients' problems concerning living with asthma or COPD. A subsequent questionnaire explicitly focused on possible research targets that aspire to solve those problems identified in the focus groups, while making final results more quantitative and representative for the entire patient community. Participants of both focus groups and questionnaire were all NAF members, selected on their willingness to participate. In many consultation studies, focus groups are

[49] This view supports the substantive argument in favour of patient participation in decision making on biomedical research, see section 1.2.2.

only used as a preliminary tool to design a subsequent questionnaire (Berry et al., 2003; Chen et al., 2001; Steine et al., 2001). However, our focus groups had the additional objective of providing insight into perspectives and arguments that underlie patients' priorities (see also Irwin, 2001).

Focus groups

In September 2003 we organized three workshops in three different regions, geographically spread across the country. Each workshop included a plenary introduction, two or three parallel focus groups, and a plenary discussion of preliminary focus group results. From the three regions, 61 patients[50] participated in seven focus groups of seven to eleven people.

All focus groups involved a majority of women (42 in total) and the average age was 56 years. More asthma patients than COPD patients were involved. Table 5.1 shows the distribution of participants among age categories and diseases. Although we originally planned to form separate groups of asthma and COPD patients, this became infeasible in practice because in two of the three regions only a few COPD patients were able to participate. Other variables were considered irrelevant for segmentation.

Table 5.1 The distribution of focus group participants along sexes, age categories, and diseases

Focus group*	Number of participants	Sex**		Age						Disease***			
		M	*F*	*0-15*	*16-30*	*31-45*	*46-60*	*61-75*	*76+*	*A*	*C*	*A+C*	*PR*
A1	7	2	5	0	1	1	4	1	0	3	2	0	2
A2	7	1	6	0	0	2	3	1	1	3	1	2	1
L1	8	2	6	0	1	3	2	2	0	4	2	1	1
L2	8	4	4	0	0	0	3	4	1	3	2	3	0
H1	11	3	8	0	1	3	4	2	1	6	2	2	1
H2	10	3	7	0	1	2	2	1	4	7	2	1	0
H3	10	4	6	0	0	1	1	5	3	2	7	1	0
Total	*61*	*19*	*42*	*0*	*4*	*12*	*19*	*16*	*10*	*28*	*18*	*10*	*5*
Total (%)	*100*	*31*	*69*	*0*	*6*	*20*	*31*	*26*	*16*	*46*	*30*	*16*	*8*

* The letters refer to the locations of the different focus groups; A = Amsterdam, L = Leusden, H = Haren.

** M = male; F = female

*** A = Asthma; C = COPD; A+C = Asthma and COPD; PR = patient relatives

[50] Since we did not distinguish different kind of NAF members, in this article the term 'patients' can refer to both actual asthma/COPD patients and relatives or carers of patients.

The focus group design was standardized and averagely structured (Morgan, 1996). Each focus group had a moderator to guide the discussion using 6 pre-established questions and exercises, and a monitor, who observed the group dynamics and recorded notes of the proceedings. Focus group discussions were recorded on video and cassette for further analysis. All participants consented to these recordings on conditions of anonymity and deletion after the project.

Each focus group session focussed on the problems patients experience in relation to their diseases and not explicitly on their research priorities. Main reasons for this are (1) talking about problems better fits in with daily experiences than talking about research priorities and (2) many patients may be unfamiliar with the full range of research topics and fields (biomedicine, social sciences, policy sciences, etc.) that could be included in an overall research agenda on asthma and COPD. In order to prevent any unintentional underexposure of some research fields or topics we deliberately avoided direct requests for research priorities. Assuming that all disease-related research eventually aims to solve disease-related problems, we argue that problems mentioned by patients indirectly refer to potential research topics or directions.

During the first part of each focus group session, patients were asked to discuss all disease-related problems they experience in daily life. In the second part of the session, patients were asked to collectively prioritize the listed problems by negotiation and distribution of urgency points. The main purpose of this prioritization exercise was to generate discussion and elicit explicit arguments that underlie each problem. Finally, each focus group identified specific research topics or questions. At the end of each plenary closing session, feedback forms were distributed that allowed participants to reflect on the workshops and to specify whether and in what ways they would like to be involved in decision-making processes in the future. Afterwards, reports of the discussions were sent to all participants for feedback.

Focus group discussions were analysed by searching the tape-recordings for mentioned causes of, and mutual relations between, identified problems. All problems, causes, and mutual relations mentioned were logically analysed in a so-called 'causal tree' (Klinkers, 2002).

In November 2003 an additional feedback meeting was conducted in order to verify the focus group results. The group of participants (27) consisted of 17 women and 10 men of whom 11 were asthma patients, 12 suffered from COPD, three from both diseases, and one was a parent of an asthma patient. The average age of these participants was 63. The main

focus of the feedback meeting was to check and complete the causal tree. For this purpose, participants were divided into three groups of eight to ten people, each of which focused on a different part of the overall causal tree. Based on the results of this meeting the causal tree was finalized.

Since people below 30 and seriously ill patients were underrepresented in both the focus groups and the feedback meeting, we held some additional in-depth interviews with younger asthma patients and a seriously ill COPD patient. We discussed their main health problems and concerns in order to determine variance with problems of the other patient participants.

Questionnaire

In order to obtain a quantitative view of patients' priorities on potential health research topics, the results of the focus groups were used to design a questionnaire. For this purpose, all problems identified in the focus groups were clustered and translated into seven categories of six potential research topics that imply the solution of the problems. An identified problem can be regarded as reflecting (1) a lack of effective solutions, or (2) a lack of adequate implementation strategies. Since health research could focus on the acquirement of knowledge for both the development and implementation of health interventions, we argue that each identified problem reflects a potential target for health research. The categories of research topics included: finances, emotions, social environment, primary care, specialist care, other forms of care, and knowledge on aetiology and drugs. These categories roughly reflect the variety of problem fields that emerged during the focus groups (see figure 5.1).

The questionnaire consisted of three blocks of questions. The first block focused on demographic characteristics of respondents. The second and main block involved the prioritization exercises, requesting respondents to divide a maximum number of points among different wishes (a variant of the 'budget pie' method, see Mullen, 1999). The third block focused on the views of respondents concerning participation in decision-making on research. In the last block, respondents were encouraged to indicate any omission in the questionnaire and to give comments.

A draft version of the questionnaire was tested by fifteen asthma/COPD patients and subsequently slightly adapted. For our analysis we needed at least 200 filled-in questionnaires. Since previous experience of the NAF indicated an average response of about 25%, we

sent the questionnaire to 1000 patients randomly selected from the entire pool of NAF members, and to 42 patients who had participated in the focus groups or the feedback meeting and had indicated a willingness to participate in a questionnaire. In addition, the questionnaire was placed on the Internet site of the NAF.

In order to check possible differences in priorities between NAF members and asthma/COPD patients who are not NAF members, we also distributed questionnaires among non-members via various hospitals, physiotherapy practices, and a respiratory rehabilitation centre in Amsterdam. Questionnaire results were analysed in a stratified way in order to identify possible influences of disease-related or demographic characteristics on research priorities.

5.2 Results

Focus groups

The results of the focus groups consisted of a causal tree of mutually related problems and causes, a list of prioritized problems, and a list of patients' questions and suggestions for research.

In figure 5.1 a simplified version of the overall causal tree of patient problems is depicted. At the highest level, there are four problem fields: psychological problems, social isolation, physical complaints, and financial problems. Problems with inadequacies of health care (both primary care and specialist care) contribute to a large cluster of problems that eventually result in physical and psychological problems. A small additional cluster is created by a lack of knowledge about the aetiology of the diseases and the lack of effective drugs, also eventually resulting in physical and psychological problems. The design of the questionnaire was based largely on these clusters of problems.

During the prioritization sessions in the focus groups, two problems consistently received more than twice as many urgency points as other problems: side-effects of medication and hypersensitivity for all kinds of substances, such as smoke, perfumes, dust, and damp. Table 5.2 lists all problems prioritized during the focus groups and their urgency scores. Problems that were added during the feedback meeting include the

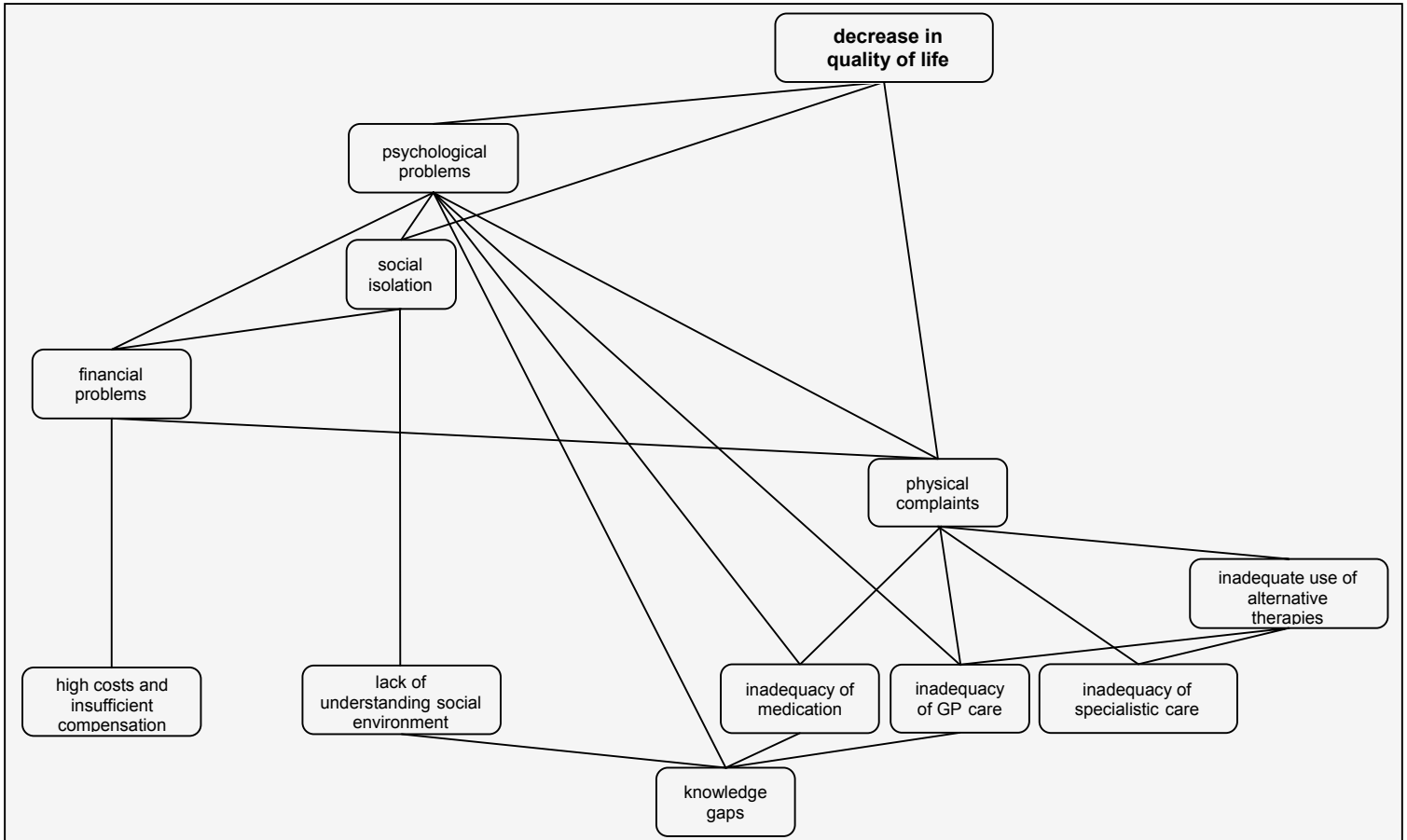

Figure 5.1. Highly simplified version of the causal tree reflecting problem (cluster)s and their mutual relations as experienced by asthma/COPD patients

lack of (deployment of) asthma nurses as coaches and mediators in health care, the lack of attention of specialists for psychological aspects of the diseases, and the non-recognition of physical causes of some complaints by medical doctors.

Table 5.2 Patients' problems prioritized during the focus g oups

Rank	*Problem*	*Score**
1	side-effects of medication	27
2	hypersensitivity for all kinds of substances, such as smoke, erfumes, dust, damp, etc.	23
3	insufficiently coaching and follow-up with drug use by profes onals	11
4	(obscurity of) long term side-effects of medication	10
5	obscurity of causes of disease or individual attacks	9
6	interference with social life	9
7	co-morbidity	7
8	inadequate information and uncertainty on drug use	7
9	fatigue	6
10	lack of knowledge among general practitioners and pharmacis s	6
11	inadequate collaboration of health care professionals	4
12	high costs for medication, aids and house adaptation	4
13	non-understanding by social environment	3
14	non-understanding at school and work	3
15	feelings of grief and frustration about physical constraints and social isolation	3
16	little attention for alternative therapies in health care	3
17	lack of patients' empowerment	2
18	inadequate collaboration between regular and alternative med ine	2
19	non-understanding by professionals	2
20	inconvenience of drug use	2

* The score refers to the number of 'urgency points' (paperclips) the problem concerned was attached to by the focus group participants

The interviews with young asthma patients indicated a common set of problems but a different rate of urgency in relation to older patients. For example, the interviewees gave higher priority to the fear of a sudden asthma attack during social activities and the insufficient knowledge of, and information from, general practitioners. The interview with the seriously ill COPD patient indicated no additional problems.

Patients' input on specific research questions mainly concerned the causes of the diseases and the occurrence of co-morbidities. Other topics included options for the improvement of treatment and the improvement of the interaction between patients and their environment. Patients' suggestions for action targeted the improvement of health care, prevention, and care organization.

On the evaluation forms, a large majority of the participants indicated that the workshop had met their expectations and that they felt they had contributed something relevant to the discussion. Main suggestions for improvement included shortening the time-scheme, using a more convenient location, providing more information beforehand, inviting a research professional who could answer questions, and realizing a better representation of male and young patients. In addition, many participants suggested that the NAF should organize similar meetings on a regular basis. Finally, 44 of the 54 patients who filled in an evaluation form indicated a willingness to be involved in the ongoing process by means of additional workshops (35), interviews (22), questionnaires (29), or participation in a committee with professionals (18).

Questionnaire

From the 1042 questionnaires sent by mail, 244 patients responded (23.4% response of which 63% was female and 36% male). In addition, six patients filled in the questionnaire from the Internet. Figure 5.2 shows the distribution of respondents according to age, education level and disease. Equal numbers of respondents suffered from asthma and COPD. Of the asthma patients, 17% regarded the degree of their disease as serious, while this was 42% among COPD patients. The median age of respondents was between 46 and 60. However, the average ages within different types of patients varied; while many respondents with COPD were above 60, respondents with asthma were often younger. Finally, most respondents had received lower secondary education but middle and higher educated people were also amply represented. Although NAF's membership includes twice as many asthma patients as COPD patients (Nederland et al., 2004), the other characteristics of our respondents roughly corresponded with the results of an earlier investigation on the demographic characteristics of NAF members (Kerklaan and Vermelis, 2002). They also corresponded with characteristics of asthma and COPD patients in general, as investigated in a national study by the Netherlands Institute for Health Services Research (Heijmans and Rijken, 2003).

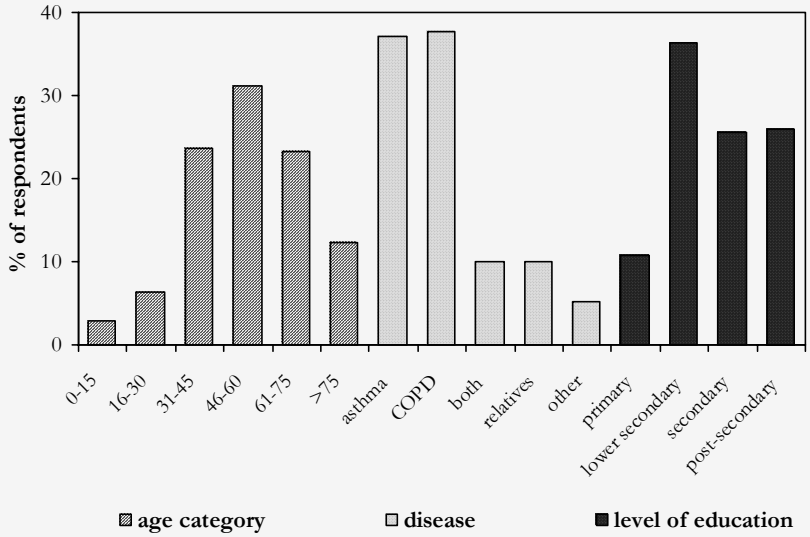

Figure 5.2 Distribution of questionnaire respondents along age categories, diseases, and levels of education.

Table 5.3 shows the 15 highest priorities of patients. Patients prioritized research on the aetiology of asthma or COPD, co-morbidity, and effective medication above research on health care or social issues. One might assume that this is the result of calculating the means since not all patients will experience, for example, problems with their general practitioners, specialists, or social environment, while they will all experience the obscurity of the origin(s) of their diseases and the inadequacy of medication. Further investigation of questionnaire results, however, showed that about 50% of the respondents prioritized biomedical issues higher than other issues on an individual basis.

A stratified analysis of questionnaire results indicated that neither sex nor age of patients significantly influenced their prioritization. Only the youngest group, the respondents below 15 years, had somewhat different priorities. This group prioritized 'Reduction of fear for hypersensitivity or symptoms during activities outside' as highest. Other high priorities of this group concerned topics that aim to improve the overall functioning of general practitioners.

Differences between the priorities of asthma patients, COPD patients and patient relatives were limited as well. All types of patients and

their relatives prioritized research on the causes of the diseases and on new medication as most important. Besides these top priorities, many asthma patients also prioritized issues related to their social environment, while seriously ill COPD patients and patients with both asthma and COPD focused on the reduction of costs.

Table 5.3 Patient priorities concerning potential research targets as resulting from the questionnaire

Rank	*Potential research target (wish)*	*Final score**
1	more knowledge on the origins of asthma/COPD	11.6
2	new and more effective drugs	11.3
3	more knowledge on the relation between asthma/COPD and other diseases (co-morbidity)	9.8
4	drugs that have less side-effects	9.4
5	more knowledge on the mutual interaction of drugs	6.3
6	the adaptation of governmental rules concerning public places and workplaces	5.6
7	more compensation for medication and aids	4.9
8	reduction of contributions for health insurances	4.4
9	improvement of understanding and consideration by family and friends	4.0
10	reduction of physical complaints that hamper daily life functioning	4.0
11	reduction of fear for hypersensitivity or symptoms during activities outside	4.0
12	compensation of costs for house adaptation	3.6
13	knowledge on effects and efficacies of alternative therapies†	3.5
14	compensation of costs for activities	3.4
15	more time and understanding by general practitioners	3.4

* Final scores refer to the average score attached to by all respondents

† Alternative therapies or complementary therapies comprise all kinds of non-traditional therapies, including homeopathy, orthomolecular medicine, yoga, breathing therapy, movement therapy, etc.

Also the level of education hardly influenced the outcomes of the questionnaire, although people with only primary education deviated somewhat in their prioritization. This group prioritized research on causes and on co-morbidity somewhat lower and asked for more attention on 'improvement of understanding by the social environment', 'the adaptation of governmental rules concerning public places and workplaces', and 'the effects and efficacies of alternative medication'.

As became apparent from the results of the additional questionnaire among non-NAF members, the priorities of NAF members did not deviate from the priorities of non-members. Apparently, the membership of the NAF does provide a bias in patients' priorities on health research.

There was some overlap between priorities identified in the focus groups and priorities that surfaced from the questionnaire. In both exercises, the side effects of medication, the obscurity of causes of diseases and symptoms, and complaints related to hypersensitivity were prioritized highly. As a difference, in the focus groups problems concerning the quality of health care, including issues of coaching, information, and coordination, received much attention, while in the questionnaire patients gave knowledge on co-morbidity and reduction of costs a higher priority.

Concerning patient participation in research in general, the majority of respondents considered patients' contributions relevant to research agenda setting and were prepared to be involved in future consultations. Most of them preferred questionnaires, but about one fourth of the respondents was willing to be interviewed or to participate in workshops or committees as well. Because of the relatively low response, the overall percentage of NAF members that are prepared to be involved in future agenda-setting processes is likely to be lower.

5.3 Discussion

In this study we aimed to sample the entire range of patients' problems concerning living with asthma/COPD. We, thus, had to be concerned whether all types of patients were represented in our focus groups – after all, different types of patients might experience different problems. Since younger patients and seriously ill patients were underrepresented, we held additional interviews. This, however, did not reveal new problems, but proved to indicate only slight differences in prioritization, as discussed above. We therefore think our focus group results adequately cover the entire range of patients' problems.

Although the response rate to our questionnaire was low, the respondents covered a representative variation in sex, disease, age, and educational level. We therefore consider our results as an adequate representation of research priorities of NAF members. In addition, since a smaller supplementary survey among non-NAF members resulted in the

same research priorities, our questionnaire results can be considered to reflect research priorities of asthma/COPD patients in general.

Differences between priorities identified in the questionnaire and the preliminary priorities that were expressed during the focus groups are probably related to differences in objectives and ways of asking between the two methods used. Whereas, for example, lack of knowledge on aetiology may not be the most obvious answer when asking for experienced problems, the acquirement of this knowledge can be considered highly relevant when presented in a list of potential research targets. This argument is substantiated by the fact that patients' research questions, as formulated during the last part of the focus groups, correspond very well with priorities resulting from the questionnaire. In addition, group dynamics within focus groups can hamper fair prioritization exercises. When discussing problems, patients will influence each other, easily resulting in potential (unintentional) over-emphasizing of a particular kind of problem and the under-exposing of other problems. When patients are confronted with a questionnaire, they can prioritize a complete series of potential research targets individually.

The patient consultation resulted in a list of priorities that reflects solutions to problems identified during the focus groups. At first sight, some of these research targets, such as those focussing on the reduction of costs or the improvement of understanding of the social environment, may seem to have little relevance for scientific research. However, we argue that all these targets could be addressed directly or indirectly by different disciplines within the broad field of health research. For example, economic research could focus on the financial aspects of diseases, while social scientific research could elaborate societal patterns of interaction between patients and their social environment.

Our results indicate that asthma and COPD patients (NAF members) prioritized biomedical research – research on the aetiology of the diseases and on new and better medication – above research on health care, social, or political issues. After comparison of the patients' priorities identified with the research priorities as formulated in the current asthma/COPD research programmes of the NAF (see box 5.1, Astma Fonds, 2000a; b), we found that patients' highest two priorities, concerning more knowledge about the causes of the diseases and more effective medication, correspond with priorities of the current NAF programme on Experimental and Descriptive Research. Priorities of the current Care and Prevention programme were addressed (and partly prioritized) by patients

when discussing problems during the focus groups, but were not prioritized as important research targets in the questionnaire.

Box 5.1 Current research programmes of the NAF*

Since 2000 the Netherlands Asthma Foundation has developed and implemented different policy programmes that together aim to realise their main policy targets. Two of those programmes include the funding of research projects: 'Experimental and Descriptive Research' (EBO – 'Experimenteel en Beschrijvend Onderzoek') and 'Care and Prevention' (Z&P – 'Zorg en Preventie').

While in the EBO programme the focus is on the development of knowledge on causes and mechanisms of asthma and COPD with the long term aim to contribute to optimal prevention and therapy in an innovative way, the focus of the Z&P programme is on the direct advancement of prevention and health care quality for asthma and COPD patients.

For the policy period 2001-2004 the research agenda for the *EBO programme* in terms of research priorities is as follows:

- Factors that influence the progression of COPD:
  - pulmonary and non-pulmonary disorders
  - heterogeneity of COPD
  - chronicity of COPD
- Insight in genetic, (psycho)physiological, and behavioural scientific aspects of smoking addiction:
  - smoking addiction behaviour
  - "(non-)susceptible smokers"
  - stopping smoking
- Extent and causes of increase in asthma:
  - chronicity of asthma
  - gene – environment interaction
  - heterogeneity of asthma
  - smoking and asthma
- Possibilities for interventions focusing on a future cure for asthma:
  - phenotyping of asthma
  - immune modulation
  - endogenous inhibition mechanisms

The agenda for the *Z&P programme* 2001-2004, in terms of targets for research, development, care innovation, and implementation projects, is as follows:

- Breaching the progression of COPD and COPD as addiction disorder:
  - better prevention of COPD
  - less underdiagnosis of COPD
  - structural embedding of quitting smoking in care and initiation of care continuum
- Prevention and better control of asthma:

- more possibilities for prevention, diagnosis and treatment of asthma among young children
- more structural embedding of self management
- better organization of care
- more preventive 'arbo'-care (concerning working conditions)

- Advancement of integral quality care
  - implementation of quality framework
  - better preconditions for quality care
  - better quality care in regions
  - more involvement of patients' perspective in care

Both programmes have been formulated in close consultation with the respective programming committees, and were based on preliminary studies on the current state of affairs concerning care and research on asthma and COPD. In 2000, the EBO programming committee consisted of 15 researchers from the biomedical, social-scientific, clinical and epidemiological fields, including the programme manager. At that moment, the Z&P programming committee consisted of 14 professionals from a variety of medical and paramedical disciplines, one patient, and a programme manager.

* (Astma Fonds, 2000a; b)

Topics that were prioritized by the patient community as third, fourth and fifth (co-morbidity, side effects of medication, and interaction between medications respectively) are not covered by the current research programmes. Possibly, research concerning the side effects of, and interaction between, medications is considered the territory of the pharmaceutical industry, and thus is not included in the NAF programmes. The fact that co-morbidity is not included in current research programmes could be explained by the strong differentiation of current medical practice and (bio)medical research; both the Dutch health care system and the biomedical research field are highly structured around individual diseases and professional disciplines. Research on co-morbidity, side effects of medication and drug interaction can thus are significant 'alternative' research priorities of patients. They originate from patients' daily experiences with their diseases and their medication and thus can be considered manifestations of patients' experiential knowledge.[51]

The priorities of patients also deviate from the priorities within the current research programmes with respect to psycho-social aspects of asthma/COPD, such as fear of hypersensitivity and the (non-) understanding by the social environment. These topics were regarded as

[51] In line with the typology of manifestations of experiential knowledge, as given in Chapter 3, these research priorities represent demands as well as judgements.

main problems by patients but are not explicitly addressed in any of the current research programmes. They also can be considered manifestations of patients' experiential knowledge.

A third difference is that patients did not focus on targets addressing smoking addiction – a priority in both NAF programmes. A possible explanation for this absence among patients' problems and priorities is that for some patients smoking addiction is a highly sensitive subject, while for others it might be irrelevant because they no longer, or never did, smoke.

5.4 Concluding remarks

The combination of focus groups and a questionnaire can be considered an appropriate methodology for investigating patients' priorities on research. The questionnaire appeared a suitable tool for explicitly consulting a representative group of patients on their research priorities, without becoming obscured by group effects. The input from focus groups was indispensable for getting a proper design of the questionnaire as well as for gaining insight in underlying arguments and perspectives.

The findings in this chapter contradict the presupposition of many people that patients are not capable of participating in broad health research agenda setting in a well-argued way. Firstly, NAF members in general seem to have sufficient knowledge to formulate and prioritize health research topics covering the entire health research field, including biomedical research. The focus group discussions have indicated that participants were able to substantiate their perspectives on priorities. Secondly, NAF members appeared to be able to think in biomedical, long-term targets and in favour of future generations, and did not only focus on individual health care and social problems. Thirdly, based on their experiential knowledge they were capable of introducing some new (biomedical) research topics that were not covered by current research agendas: co-morbidity, side effects of medication, and drug interaction. One could reasonably assume that other patients will be able to do the same.

Based on this study we thus conclude that patients are capable of participating in health research agenda setting in a well-argued way. Indeed, although just as medical professionals patients have their own biases, they have something new to contribute to research agendas, which

pleads for their participation in health research agenda setting. Since biomedical research was part of the agenda-setting process and since patients proved to be able to introduce and prioritize biomedical research topics, we argue that patients are capable of participating in biomedical research agenda setting as well.

6

A SOCIAL EXPERIMENT[52]

This chapter reports on a social experiment that aimed to implement and evaluate a newly developed strategy for effective patient participation in research agenda setting, which has been described in chapter 4. The experiment involved an interactive agenda-setting project concerning research on asthma and COPD commissioned by the Netherlands Asthma Foundation (NAF) and the Netherlands Organization of Health and Medical Research (ZonMw). In this project, patients were, besides scientists and health care professionals, involved in decision-making on an overall asthma/COPD research agenda. The project design consisted of the first four phases of the developed strategy: preparation, consultation, collaboration, and prioritization. Whereas the consultation of patients has been described in detail in the previous chapter, this chapter describes the remaining part of the project. The project resulted in an integrated 'societal' research agenda that reflected the priorities of all stakeholders involved and was meant as an input for further research policy making by the NAF and ZonMw. The effectiveness of the participation exercise was assessed on the basis of an evaluation framework. The evaluation results suggest that the strategy used had realized rather effective patient participation, with respect to the legitimacy and rationality of the process, the quality of the outcomes, and the achievement of mutual learning. Patients had been involved in a fair and direct way and they had visibly influenced the resulting societal research agenda. In addition, mutual learning between the different stakeholders had been achieved to some extent.

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[52] The text of this chapter is partly based on Caron-Flinterman, J.F., Broerse, J.E.W., and Bunders, J.F.G. (submitted). Patient participation in health research agenda setting: The case of asthma and COPD research in the Netherlands. *Science and Public Policy*.

The main aim of this chapter is to describe the implementation and evaluation of the patient participation strategy developed in chapter 4 in a concrete practical situation. The implementation of the strategy took place within the framework of a recent project that concerned the participation of patients in agenda setting of asthma/COPD research in the Netherlands. This agenda setting concerned the entire breadth of health research and was not restricted to biomedical research. However, since biomedical research was explicitly part of the agenda-setting process, we considered the project an appropriate case for testing our strategy. We start this chapter with a description of the participation initiative and its outcomes. In order to be able to evaluate this initiative in terms of effectiveness, we formulate a framework based on the objectives of patient participation as defined in section 1.2.2. We subsequently present the evaluation results and close with some concluding and reflective remarks and some suggestions for the improvement of patient participation strategies.

6.1 The interactive research agenda-setting project[53]

As has been mentioned before, the interactive[54] research agenda-setting project was commissioned by the Netherlands Asthma Foundation (NAF) and co-financed by the Netherlands Organization for Health Research and Development (ZonMw). The NAF is both a research-funding agency and a patients' organization focusing on asthma and Chronic Obstructive Pulmonary Disease (COPD). At the moment, it funds different types of

[53] The text of this section is based on: Teerling, J., Caron-Flinterman, J.F., and Broerse, J.E.W. (2004). Programmering wetenschappelijk onderzoek astma en COPD 2005-2008: De maatschappelijke agenda. Amsterdam: Instituut voor Innovatie en Transdisciplinair Onderzoek, Vrije Universiteit Amsterdam.

[54] In literature one often uses the terms 'participative' or 'participatory' instead of 'interactive' when describing comparable strategies or initiatives (e.g. Andersen and Jaeger, 1999; Black and Gergersen, 1997; Cornwall and Jewkes, 1995; Gray et al., 2000). In this project, we use the term 'interactive' in order to stress that in our initiative patients not only participated in the process (which could imply different degrees of participation) but actually interacted with other participants in a sphere of true partnership and knowledge integration (cf. Tress et al., 2003).

health research within the context of two policy programmes (see box 5.1). The Experimental and Descriptive Research (EBO) programme focuses on knowledge production concerning the causes and mechanisms of asthma and COPD, with the long-term aim of innovatively contributing to optimal prevention and therapy (Astma Fonds, 2000a). The Care and Prevention (Z&P) programme commissions only applied research focusing on the direct advancement of prevention and health care quality for asthma/COPD patients (Astma Fonds, 2000b). ZonMw, the national medical research council in the Netherlands, also funds some applied research on asthma or COPD within their Health Promotion and Disease Prevention Programme.[55]

Until recently, the NAF had used an expert-led approach to research programming, inviting leading scientists and health professionals to advice on research priorities through series of discussion and prioritization meetings. At the end of 2002, the NAF decided to involve patients in decision-making on overall health research agendas for the period 2005-2008. Since ZonMw also funds research on asthma/COPD, the NAF sought co-operation with ZonMw in order to assess possibilities for collaboration and mutual gearing of research policies and programmes.

Because of our previous experiences in developing and implementing participatory strategies, the NAF consulted our Institute[56] for the purpose of realizing patient participation in research agenda setting. As a result, in February 2003 we started an interactive research agenda-setting process. This process comprised the implementation of the first four phases of the phased transdisciplinary participation strategy that was developed in chapter 4. The strength of this strategy lies in the structured way in which consultation and collaboration methods are combined and in the explicit focus on the stimulation of knowledge integration. The design of the different phases and the (intermediary) results are described in more detail below.

6.1.1 Preparation and initiation phase

From February to July 2003, we conducted some preliminary research on the current situation of asthma/COPD research in the Netherlands. By means of fourteen semi-structured interviews with relevant actors in the

[55] www.zonmw.nl

[56] the Athena Institute for Research on Innovation and Communication in Health and Life Sciences, Faculty of Earth and Life Sciences, Vrije Universiteit Amsterdam

field (researchers and representatives of funding agencies and industry) and additional desk studies, current decision-making patterns were investigated and visions of stakeholders concerning patient participation in decision-making on research were explored. Results indicated that until then patients were hardly involved in decision-making processes concerning research on asthma or COPD. Most stakeholders interviewed considered patient participation in decision-making on asthma/COPD research in principle desirable, but at the same time identified some obstacles that hamper effective participation. Obstacles mentioned mainly concerned the incompetence and subjectivity of patients and the unwillingness of researchers to translate research topics and proposals into daily language. Identified conditions for effective patient participation in research agenda setting concerned the improvement of communication between patients and researchers, the education of patients on research items, and an important and facilitating role of the NAF (De Cooker and Koningstein, 2003).

Based on our previous findings and earlier experiences, we regarded none of the obstacles identified as insurmountable and considered an interactive stakeholder approach feasible. Subsequently, the NAF and ZonMw commissioned the Athena Institute to execute an interactive research agenda-setting project entitled 'Interactive programming of asthma /COPD research: the societal agenda'. This project formally started July 2003 and ended June 2004. The formal part of the project consisted of the phases 2, 3, and 4 of the proposed strategy. The objectives of the project, the different stakeholders to be involved, the respective roles of the NAF, ZonMw, and the project team, the overall process structure, and the composition of the project team were defined and described in a project document and formal contract. The NAF and ZonMw jointly financed the project and a total budget of 56,000 Euros was made available.

The main, substantive objective of the project was the construction of a 'societal' agenda for research on asthma or COPD that reflected the perspectives of the three main stakeholders of this research – patients, scientists, and health professionals. Another, more normative-based objective for the NAF and ZonMw was the enhancement of the legitimacy (and thus the social support) of the agenda-setting process and of subsequent research policy. An additional scientific objective for us was to realize and investigate mutual learning and knowledge building. This could result in an increased insight in knowledge integration processes, which

may contribute to knowledge production on, and the further optimization of, stakeholder participation processes.

The design, management and execution of the agenda-setting project were predominantly the responsibility of the project team. This team consisted of (1) four staff members of the Athena Institute (including the author of this dissertation), who were responsible for the design, execution, and analysis of the interactive process, (2) three MSc students who assisted in the project as trainees, and (3) six MSc students who were involved in the organization and execution of the focus groups with patients (as part of a course on interactive research methodologies). The roles of the NAF involved the funding of the project, reflection on various aspects of the process design, and the facilitation of both the recruitment of adequate stakeholder representatives and the distribution of invitations, information, and the questionnaire. In addition, one of the NAF managers investigated the current status of scientific research concerning asthma or COPD. The roles of ZonMw remained restricted to funding and some reflection on process design.

6.1.2 Consultation phase

During the period September 2003 until March 2004, the three stakeholder groups (patients, scientists, and health professionals) to be involved in the interactive research agenda setting were explicitly and directly consulted on their priorities concerning research on asthma or COPD. Since the three stakeholders considerably differed with respect to their experience with research-agenda setting processes and their background knowledge on asthma/COPD research they were consulted by means of different consultation methods. The consultation included both inventory studies that aimed to explore views and perspectives of stakeholders on research priorities in a broad way and more in-depth studies that aimed to elaborate on the views and perspectives identified in order to get a more thorough understanding of the research priorities of the different stakeholders as well as of similarities and differences between these priorities.

Throughout the process all participants received information about the process background, objectives, and structure, as well as on objectives and programmes of individual meetings or interviews. When patients were involved, scientific or professional terms were translated into daily language. All meetings were carefully chaired and facilitated by experienced project team members, who stimulated mutual respect and learning. In

addition, the meetings were recorded in minutes and on video or cassette for further analysis of underlying arguments, mutual interactions etc. All participants consented to these recordings on conditions of anonymity and deletion after the project. Interviews were conducted by both staff members and trainees. After each interview or meeting, interviewees or participants involved received a report with the request to provide comments if misinterpretation or omissions were found. In this way, interpretations and intermediary results could be verified. Below we describe the consultation of the different stakeholder groups.

Patients

For pragmatic reasons and in consideration with the NAF, patient consultation was restricted to NAF members. Since at that time the NAF did not have a pre-existing group of organized patients that could be consulted initially (during a reorganization operation in 2000 the Patient Advisory Board (PAR) was abolished), we turned to the broad group of NAF members. For the consultation of these patients[57] we decided to start with a number of focus groups followed by a questionnaire.[58] The patient consultation step and its results have been described in detail in chapter 5.

Health care professionals

Research priorities of the broad range of health care professionals concerned with asthma or COPD were initially explored in two discussion meetings with 11 members of the programme committee Care and Prevention (see box 5.1) of the NAF. This committee covers a wide range of disciplines and all members have good insight into the current state of affairs concerning care around asthma or COPD. The discussions had partly an exploratory character, focusing on identified problems (suboptimal situations) in health care and on priorities for improvement, and partly an in-depth character, focusing on mutual relationships between problems, causes, and arguments for priorities. All problems and causes were incorporated in a causal tree, which could be linked to the causal tree of patients' problems (see figure 5.1).

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[57] In the remaining part of this chapter we use the term 'patients' to refer to the entire width of NAF-members, which includes partners, family members and carers of patients as well.

[58] This combination of the focus group and questionnaire methods has been previously used e.g. in consulting the public on their views and priorities concerning developments within the biosciences (Irwin, 2001).

In 13 subsequent semi-structured interviews, both with committee members and with additional health care professionals, this causal tree was further specified and supplemented. Interviewees were selected on the basis of their (expected) central position within a discipline, while all health care disciplines involved with asthma or COPD were covered. In addition, problems identified were translated into possible research objectives that were prioritized by all interviewees. From each interviewee the three highest priorities were included in a final list of priorities (see table 6.1).

Table 6.1 Research priorities of health care professionals*

-	better coordination of health care
-	early diagnosis and prognosis of asthma
-	societal attention for COPD
-	better tuning between health care demand and supply
-	genetic predispositions of asthma and COPD
-	better treatment of COPD
-	patient empowerment
-	better diagnosis of COPD
-	prevention of smoking
-	introduction of case managers for asthma patients
-	roles and functions of asthma nurses

* The order of the topics does not reflect any order of priorities.

Scientists

A preliminary inventory study by one of the research programme managers of the NAF had resulted in a list of knowledge gaps and research opportunities in scientific research on asthma or COPD. This list was used as an input for a discussion meeting with 16 scientists from different scientific disciplines, which had been selected by the programme manager on the basis of their central position within a discipline.[59] It was attempted to cover all scientific disciplines involved with research on asthma or COPD. Although both (bio)medical and socio-cultural scientists had been invited, only (bio)medical scientists were able to attend the meeting. Several of them were members of the EBO programme committee (see

[59] In the expectation that these scientists might be able of representing their disciplines in a quite adequate way since they may have good overview of current research directions and priorities within that disciplines.

box 5.1) and thus were used to being involved in research agenda setting. During smaller group discussions these scientists were asked to mention and discuss priorities concerning scientific research on asthma or COPD. In subsequent exercises they were asked to find consensus on the highest priorities. They finally agreed on a list of eight (bio)medical research priorities, which is depicted in table 6.2.

Table 6.2 Research priorities of (bio)medical scientist *
- early disease markers for, and phenotyping of, asthma and COPD
- aetiology of, and mechanisms for remission and persistence of asthma
- longitudinal psychosocial, diagnostic, and mechanistic aspects of COPD
- translation of *in vitro* results to disease-specific complex cell culture systems and *in vivo* models
- individual treatment of asthma and COPD on the basis of phenotype and proof of concept studies on long-term disease modifications

* The order of the topics does not reflect any order of priorities

Since socio-cultural scientists were not present during this discussion meeting, this group of scientists was consulted by means of five semi-structured interviews. Interviewees were selected on their expected adequate representation (recommended by the NAF or by colleague scientists) of the few socio-cultural scientific research groups focusing on asthma or COPD. The highest research priorities of these interviewees were incorporated in a fourth list of research priorities (see table 6.3).

The consultation phase thus resulted in four lists of (possible) research priorities (tables 5.3, 6.1, 6.2, and 6.3), which partly overlapped. For example, all stakeholder groups mentioned additional research on the aetiology of asthma/COPD as a research priority, and also research on better medication for, and early diagnosis of, asthma/COPD scored high. Research priorities of patients that were not covered by the other stakeholder groups concerned research on co-morbidity, drug interaction and side effects, reduction of (medical) costs, and improvement of societal understanding and consideration.

Table 6.3 Research priorities of socio-cultural scientis*
- better transitions between care and daily life
- patient empowerment
- aetiology of asthma and COPD (both genetic and environmental factors)
- smoking behaviour
- societal attention for COPD
- early diagnosis of COPD
- better diagnosis of asthma amongst young children
- prevention of asthma
- behavioural aspects of asthma and COPD
- relations between labour circumstances and the onset, increase, c decrease of asthma/COPD
- influence of psycho-social circumstances on children with asthma
- more integral health care

* The order of the topics does not reflect any order of priorities

6.1.3 Collaboration phase

The aim of the collaboration phase (March and April 2004) was the explicit integration of the four priority lists into one shared research agenda by representatives of the different stakeholder groups.[60] For this purpose it was decided to organize an integration meeting to discuss perspectives and priorities of different stakeholders, to accomplish mutual respect, understanding, and learning between the different stakeholders, to integrate the different research priorities into one research agenda, and to identify criteria for further prioritization of the research agenda.

The integration meeting took place on 2 April 2004 in Utrecht. Of the 32 accepted invitations (we aimed for roughly 10 participants from each stakeholder group), one biomedical scientist, four health care professionals, and three patients were unexpectedly unable to attend the meeting. The 24 participants included six (bio)medical scientists, four

[60] Since both professional stakeholder groups consulted included members of the formal NAF programme committees (whose advices on research programmes were usually taken up by the NAF management), this integration of priority lists can be considered as the integration of patients' priorities with the priorities of central actors of the decision-making network, which corresponds to the term 'collaboration' as used in patient participation literature.

socio-cultural scientists, five medical specialists/clinical researchers, one other health care professional, and eight patients (three asthma and one COPD patients, two patients with both asthma and COPD, and two parents of young asthma patients). Scientists and health care professionals were selected based on the diversity of their background and on their indicated willingness to share decision-making with patients. Patients were selected from the focus group participants based on their indicated wish to attend an interactive meeting and on their capability to express themselves clearly and constructively as assessed during the focus groups. Before the meeting all participants received a summary of the consultation phase and its results by mail. Scientific or professional terms were translated into daily language.

The meeting started with a plenary session in which the results of the consultation phase were presented and the aim of the meeting was specified. Then, the participants were split into three parallel, heterogeneous groups that discussed the four priority lists and tried to categorize and integrate the four lists into one integral research agenda. For this purpose all priorities were described on coloured post-its (the used colours depended on the stakeholders that had selected the priorities) that could be categorized and re-categorized on flip charts until consensus was achieved. After that the participants were individually asked to define criteria that could be used for further prioritization of this research agenda. Criteria were gathered on flipcharts and discussed and categorized as well. In a plenary session the integrated and categorized lists of research topics and the lists of criteria from the different groups were presented and discussed. Since the lists showed much similarity, they could be combined rather simply into one integrated 'societal' research agenda (see table 6.4), and one list of prioritization criteria (see table 6.5).

Table 6.4 The 'societal' agenda concerning research on asthma or COPD.

Themes and research targets	*Priority**
Insight in causes and mechanisms of asthma and COPD	
- research on genetic factors causing asthma	P
- research on genetic factors causing COPD	P
- research on environmental factors and lifestyles that influence the onset of asthma	P
- research on environmental factors and lifestyles that influence the onset of COPD	P
- research on causes and mechanisms of increase or decrease of asthma symptoms	P

- research on the course and mechanisms of COPD during life	P
- research on possibilities for translating results of molecular and cel lar research to disease related complex cell culture systems and animal models	
Insight in causes and mechanisms of co-morbidities	
- research on relations between asthma and other diseases	
- research on relations between COPD and other diseases	P
Improvement of prevention of asthma/COPD	
- research on smoking behaviour and on interventions that influence starting and stopping with smoking	P
- research on interventions that prevent the onset of asthma	P
- research on interventions that prevent the onset of COPD	P
Improvement of diagnostics for asthma/COPD	
- research on the earliest stages of asthma and on methods to detect hese stages	P
- research on the earliest stages of COPD and on methods to detect hese stages	P
- research on improving the diagnosis of asthma based on individual lisease characteristics	
- research on improving the diagnosis of COPD based on individual lisease characteristics	
- research on diagnostic aspects of COPD during different stages of he disease	
Improvement of treatment of asthma/COPD	
- research on improving the treatment of asthma based on individua disease characteristics	P
- research on improving the treatment of COPD based on individua disease characteristics	P
- research on new, more effective therapies for asthma	
- research on new, more effective therapies for COPD	
- research on side-effects of asthma/COPD medication	
- research on the interaction between asthma/COPD drugs and oth drugs	
Improvement of care organization	
- research on possibilities for better tuning of care processes to the d ily life of the patient	
- research on possibilities for better streamlining of health care for th patient	
- research on possibilities for better gearing of care demand and sup y	
- research on improving care for COPD patients during the latest st ges of the disease	
Improvement of patient empowerment	
- research on factors that improve the empowerment of the patient t wards emancipated care user	
Improvement of accessibility of care	
- research on the desirability of, and possibilities for, reducing health nsurance contributions	
- research on the desirability of, and possibilities for, increasing finar ial compensation for medication and aids	
- research on cost-effectiveness of therapies and aids	

Improvement of interaction between asthma/COPD patients and heir environment	
- research on the influence of (psycho-)social circumstances on child en with asthma	P
- research on the interaction between asthma/COPD patients and th ir closest environment and on possibilities to improve this interaction	
- research on the relation between labour circumstances and the ons , increase, or decrease of asthma	
- research on the relation between labour circumstances and the ons , increase, or decrease of COPD	
- research on the desirability of, and possibilities for, adaptation of le islation concerning smoking, ventilation, etc. in public spaces and at worki g places	
- research on possibilities to improve the familiarity of, and attentior for, COPD at the public and the government	
Insight in personal impacts of asthma/COPD	
- research on psychosocial aspects of COPD related to the course of he disease during life	
- research on psychosocial aspects of asthma	
- research on (reduction of) fear for hypersensitivity or physical com laints among asthma/COPD patients	

* P = belongs to the 15 highest prioritized research topics

Table 6.5 Priority setting criteria

Criteria with respect to the purposefulness of the research
- the research addresses a relevant knowledge gap in health care or research
- the research addresses an important problem for patients or care profession s
- the research addresses a core problem that causes many other problems
- the problem addressed is considered urgent and requires instantaneous actic n
- the problem addressed concerns a big population
- the research addresses a problem that costs a lot of money and attention
- the research addresses a cost saving
Criteria with respect to the efficiency of the research
- the research could result in a large theoretical spin-off
- the research results could have a direct impact on patient's quality of life
- the research seems to provide short term results
- the research builds on existing knowledge
- the expected results are favourable related to the expected investments
- the research is feasible with current research methods and tools
- the problem can be addressed by different types of research

6.1.4 Prioritization phase

A final exercise in the project aimed to identify priority research topics within the agenda. In order to prevent bias (pseudo consensus) in the prioritization process, due to group dynamics and power or status differences, selected participants were asked to prioritize the research agenda individually. For this purpose we used a 'prioritization matrix'. In the matrix, vertically the overall health research agenda with its research topics was listed while horizontally respondents were asked to rate the different research topics of the agenda by giving marks and to indicate in the matrix which criteria determined their priority setting. The prioritization matrix was sent to all earlier consulted scientists and professionals (39) and all patients who had indicated before to be willing to participate in dialogues with professionals (23). Eight (bio)medical scientists, six socio-cultural scientists, six medical specialists/researchers, six other health care professionals, and fifteen patients (eight asthma patients, three COPD patients, two patients with both asthma and COPD, and two parents of asthma patients) returned a filled in matrix (66% response).

Final results were achieved by collecting the priorities of the different participants (the research topics that they had given the three highest marks). The frequencies with which research topics had been prioritized by individual participants determined the overall priorities of the topics. The total scores (added marks) of research topics were used to discriminate priorities of topics with similar frequencies of prioritization. The highest prioritized research topics were investigated in terms of the different criteria ascribed to. The fifteen highest prioritized research topics are indicated in the right hand column in table 6.4. These topics concern research that focuses on causes and mechanisms, prevention, diagnosis, and treatment of asthma or COPD, except one topic that comprises research on the influence of psycho-social circumstances on children with asthma. The three highest priorities of the different stakeholders during this priority setting exercise are presented in table 6.6.

The three stakeholder groups used the selected criteria for further priority setting of research topics in a more or less similar way, although patients in general did ascribe fewer criteria to the different research topics than professionals. Nearly all research topics of the research agenda were indicated to meet the criteria 'addresses a relevant knowledge gap' and 'concerns a big population'. In addition, the twelve highest prioritized

Table 6.6 Priorities of different stakeholders during final priori y setting

Patients
- research on environmental factors and lifestyles that influence the onset of a thma
- research on the relation between labour circumstances and the onset, incre se, or decrease of asthma
- research on causes and mechanisms of increase or decrease of asthma symp oms

(Bio)medical scientists
- research on the earliest stages of COPD and on methods to detect these sta es
- research on smoking behaviour and on interventions that influence startin ; and stopping with smoking
- research on interventions that prevent the onset of COPD

Socio-cultural scientists
- research on psychosocial aspects of COPD related to the course of the disea e during life
- research on psychosocial aspects of asthma
- research on environmental factors and lifestyles that influence the onset of a thma

Medical specialists/researchers
- research on smoking behaviour and on interventions that influence startin ; and stopping with smoking
- research on causes and mechanisms of increase or decrease of asthma symp oms
- research on the earliest stages of COPD and on methods to detect these sta es

Other health care professionals
- research on environmental factors and lifestyles that influence the onset of C OPD
- research on smoking behaviour and on interventions that influence startin ; and stopping with smoking
- research on the earliest stages of COPD and on methods to detect these sta es

topics scored high on 'might result in a large theoretical spin-off', 'builds on existing knowledge', 'addresses a core problem that causes many other problems', and 'can be addressed by different types of research'. Two criteria that patients ticked more frequently than the other stakeholders were 'the results may have a direct impact on patient's quality of life' and 'addresses an important problem for patients or care professionals'.

The project activities and results were published in a report (Teerling et al., 2004; in Dutch) and presented during two meetings of the NAF: the yearly general meeting in June 2004, attended by NAF-members and – staff members, and an invitational conference on NAF's future policy in September 2004, attended by a range of different stakeholders that have a stake or interest in this policy. The resulting societal research agenda is currently used as guideline for the formulation of research policies and programmes of both the NAF and ZonMw, which will

eventually result in the actual funding of research projects as well as in possible lobbying activities towards pharmaceutical industry, government, or other actors. This specification and implementation (phases 5 and 6 of the proposed strategy) are in the hands of the management of both NAF and ZonMw and were not included in the project.

6.2 An evaluation framework

But to what extent has the participation strategy followed resulted in an effective participation process? Many scholars have formulated evaluation criteria for effective stakeholder or public participation in decision-making. They have based their frameworks on the views of participants (e.g. Bickerstaff and Walker, 2001; Carnes et al., 1998; Morrissey, 2000; Telford et al., 2004) or on implicitly or explicitly adopted definitions of effective participation. As has been argued in section 2.3, these definitions refer to objectives of participation, which can concern the participation process itself as well as its outcomes. As a consequence, evaluation frameworks focus on the participation process (e.g. Fiorino, 1990; Halvorsen, 2003; Laird, 1993), on its outcomes (e.g. Beierle and Konisky, 2000; Guston, 1997; 1999; Irvin and Stansbury, 2004), or on both (e.g. Abelson et al., 2003; Driessen et al., 2001; Rowe and Frewer, 2000; Webler, 1995; Webler and Tuler, 2000).

In line with the suggestions of Rowe and Frewer (2004), we formulate a suitable framework for the systematic evaluation of our participation strategy, starting with defining 'effectiveness' of patient participation on the basis of its objectives. In section 1.2.2 we elaborated on the different objectives of patient participation in decision-making on biomedical research. Within the context of patient participation in asthma/COPD research agenda-setting, important objectives are the legitimacy and rationality of the agenda-setting process and the quality of the resulting research agenda. These objectives had been more or less adopted by the NAF and ZonMw as well. The 'quality' of the research agenda was defined as its usefulness and adequate reflection of the perspectives of the three main stakeholders of asthma/COPD research. The social acceptance of the resulting agenda is not a very obvious objective of this specific agenda-setting process, because of its non-controversial and non-public character. Besides, the acceptance of the agenda by NAF-members, NAF-supporters, researchers, and other

relevant stakeholders will mainly depend on both the legitimacy of the process and the quality of the outcomes. Therefore we left this aspect out of our evaluation framework. Increases in human and social capital were neither included in our framework since these both concern long-term outcomes that are very difficult to measure shortly after the exercise and depend on more factors than the participation strategy followed. Instead, we included the extent of mutual learning as an objective, since this is a precondition for increases in both human and social capital. In addition, a closer investigation of mutual learning could result in an increased insight in knowledge integration processes, which may contribute to knowledge production on, and the further optimization of, stakeholder participation processes.

Based on these objectives, we thus consider patient participation in research agenda setting effective when procedures followed are legitimate and rational, when the resulting research agenda is useful and adequately reflects the perspectives of patients, and when mutual learning between patients and professionals has been accomplished. Consequently, our evaluation framework should consist of both process and outcome criteria that focus on the achievement of these objectives.

Concerning the existing frameworks that focus on both the process and its outcomes, we found none of them sufficiently adequate for evaluating our participation strategy in a conclusive manner. Criteria mentioned are not sufficiently detailed (Driessen et al., 2001) or discriminating (Abelson et al., 2003), do not sufficiently reflect our definition and objectives of participation, and/or do not entirely apply to our context of patient participation in research agenda setting (Irvin and Stansbury, 2004; Rowe and Frewer, 2000; Webler and Tuler, 2000). We therefore borrow criteria from all these frameworks in order to formulate a new and more adequate evaluation framework below.

6.2.1 Process criteria

Process criteria for effective patient participation in research agenda setting need to reflect the agenda-setting legitimacy and rationality. Agenda-setting legitimacy can be achieved by involving patients in a representative and fair way, both in preliminary discussions on alternatives and criteria, and in final decision-making processes. In this way the process gives judgment to normative values underlying patient participation. Agenda-setting rationality can be achieved by ensuring that patients' values, experiences,

and knowledge are included in discussions and decision-making processes. Key elements of the process to be evaluated are the stakeholder representation in the process, the process structure, and the process management.

- *Stakeholder representation:* In order to achieve legitimacy, equal numbers of representatives from all key stakeholder groups should participate in decision-making steps of the process. Concerning patients, numbers of representatives should be higher rather than lower than the numbers of professional participants (Laird, 1993). In this way power imbalances due to unequal attendances are avoided. In addition, participants should adequately represent the different stakeholder communities (Abelson et al., 2003; Rowe and Frewer, 2000; Webler and Tuler, 2000). This implies the involvement of balanced representations of the demographic characteristics of the relevant patient community and of the spectrum of relevant professional disciplines.
- *Process structure:* An effective participation process needs to be clearly and transparently structured (Rowe and Frewer, 2000). All participants need to know and understand which process objectives and roles of actors are involved, which steps the process consists of, how information is (or has been) gathered and processed, how decisions are (or have been) made, etc. (Rowe and Frewer, 2000; Webler and Tuler, 2000). In addition, both process structure and procedures used should ensure that patients are directly consulted for their views, priorities, needs, etc. as early as possible in the process (Abelson et al., 2003; Rowe and Frewer, 2000; Webler and Tuler, 2000). Moreover, in order to ensure the rationality of the agenda-setting process, procedures used need to ensure that patients are directly involved in on-going discussions and decision-making steps, and that their input is explicitly incorporated in the outcome (Driessen et al., 2001; Fiorino, 1990; Laird, 1993; Webler and Tuler, 2000).
- *Process management:* In order to ensure agenda-setting legitimacy, process managers and facilitators should be independent from parties involved and indifferent with respect to the outcomes of the process (Rowe and Frewer, 2000). Throughout the process, participants need to be and feel treated equally by the process management and have equal access to information and other resources such as payment of expenses (Abelson et al., 2003;

Fiorino, 1990; Laird, 1993; Rowe and Frewer, 2000; Webler and Tuler, 2000). In addition, during the various meetings, the process management should strive for an open, and respectful atmosphere that stimulates constructive interaction and mutual learning between participants (Abelson et al., 2003; Driessen et al., 2001; Fiorino, 1990; Halvorsen, 2003; Laird, 1993; Webler and Tuler, 2000).

6.2.2 Outcome criteria

Criteria concerning the outcomes of the interactive health research agenda setting process need to evaluate both direct and indirect outcomes. Direct process outcomes concern resulting research agendas and other relevant decisions. The indirect outcome to be evaluated here concerns the achievement of mutual learning between participants.

- *Direct outcomes:* The resulting research agenda should be based on consensus among participants (Abelson et al., 2003). In addition, it should reflect the inputs and perspectives of patients as well as of other stakeholders involved (Beierle and Konisky, 2000; Driessen et al., 2001; Irvin and Stansbury, 2004). All participants should recognize their own perspectives in the outcome. Furthermore, the research agenda needs to be reasonable and well translatable into future policy or actions. If necessary, participants should be able to explain and substantiate outcomes (Abelson et al., 2003).
- *Indirect outcomes:* An indirect outcome that characterizes the effectiveness of our patient participation initiative is mutual learning resulting in changes in thinking of participants. This implies the learning of both patients and professionals in a substantive way (concerning substantial matters), in a procedural way (concerning participation procedures and methods), or in a reflexive way (concerning their own and each others knowledge, perspectives, roles, etc.) (Guston, 1999; Irvin and Stansbury, 2004). The occurrence of this kind of knowledge sharing between participants might facilitate future participation processes.

In table 6.7 the evaluation criteria of our framework are summarized.

Table 6.7 Evaluation criteria for effective patient participation in he alth research agenda setting			
Process criteria	Stakeholde	representation	adequate and equal representation of takeholders
	Process sti	ıcture	transparency of objectives, roles and tructure
			early & direct involvement of patient
			explicit incorporation of patier s' inputs in discussions and decisions
	Process m	nagement	independent and unbiased manageme nt
			equal treatment of participants
			facilitation of mutual respect, penness, and constructive interaction
Outcome criteria	Direct pro	ess outcomes	consensus on resulting research agen a
			reflection of patients' perspectives in esearch agenda
			practicality of resulting research agen a
	Indirect ou	comes	achieved (mutual) learning on substa tive matters
			achieved learning on procedural mat rs
			achieved (mutual) learning on reflect e matters

6.3 Evaluation of the project

The project was evaluated in terms of effectiveness based on the evaluation framework described above. Data concerning the evaluation of the effectiveness of patient participation within the project had been collected by means of a triangulated approach, involving documentary data analysis, video and cassette tape analysis, (direct) observation, and semi-structured interviews, which were held within three months after the integration meeting. Meeting scenarios, minutes, and reports provided initial information on the adequacy of the overall process structure and the procedures used. Analysis of feedback reports and evaluation forms provided additional information on participants' opinions concerning the adequacy of the procedures used, the analysis of results, etc. Comparison of the various reports provided insight in the actual influence of the different stakeholders on the resulting research agenda.

Observation and tape analysis were used to investigate additional aspects of the process more thoroughly, such as the equality among, and interaction between, participants and their actual inputs in discussions. Although observation holds the danger of leading to subjective and biased results, it also offers important research opportunities that might be

missed when restricting to more objective and retrospective evaluation methods only. Semi-structured interviews with all participants who had been involved in the integration meeting (and thus in the entire agenda-setting process) were used to investigate visions of participants on both the process and its outcomes more thoroughly. Interviews with two NAF managers, finally, provided amongst others information on the practicality of the outcomes. All interviews were held in the period April to June 2004. Interview reports were returned to the interviewees for feedback.

Different members of our project team were involved in different parts of the evaluation process. Three project team members (including the author of this dissertation) were responsible for the design of the overall evaluation process, and were involved in both documentary data analysis and observation. The three MSc trainees, who had assisted in the execution of the participation project but had not been involved in process design and management, conducted the evaluative interviews. The triangulation of both methods and investigators enhanced the validity of the results. Below we describe the findings of the evaluation process. We start with evaluating the process itself, followed by an evaluation of the process outcomes. When possible and relevant we have illustrated our findings with citations from the interviews.[61]

6.3.1 Evaluation of the process

Adequate representation of stakeholders

Three stakeholder groups participated in the agenda-setting process: patients, health care professionals, and scientists. During the consultation phase, respectively thirteen biomedical scientists, six socio-cultural scientists, eight medical specialists/researchers, and twelve health care professionals were involved, representing the main disciplines involved in asthma or COPD research or care. In addition, more than 300 patients, that together reflect the demographic and disease-related characteristics of the entire NAF member community as well as the Dutch population of asthma/COPD patients in general, were consulted. In this way we achieved an adequate representation of Dutch asthma/COPD patients. We thus can conclude that during the consultation phase an adequate representation of stakeholders had been achieved.

[61] The original citations were in Dutch and have been translated by the author.

During the integration meeting we had intended to have equal numbers of patients, health care professionals and scientists. However, because of late excuses in particularly the group of patients and health professionals we did not succeed in this respect. This also implied that some professional disciplines were missing (a General Practitioner, a physiotherapist, a child psychologist, an asthma nurse, and a pharmacologist) and that the number of COPD patients was relatively low. Several interviewees specifically mentioned these shortcomings in representation:

> "My group mainly consisted of scientists and health care professionals/researchers. To my opinion more pure health care professionals could have been present. I think it was a fair process, but the balance in representation was somewhat distorted. More stakeholders should have been involved, such as asthma nurses, social workers, etc." (a social scientist)

> "I think more people from the sociological field should have been present." (a medical specialist/researcher)

> "I think there were more biomedical scientists present. However, I do not think outcomes would be different when stakeholder representation was more balanced because all perspectives and views have been discussed." (a biomedical scientist)

In addition, interviewees mentioned that the number of patient participants was rather low in comparison with the total number of 'expert' participants. Another comment with reference to the patient participants in the collaboration phase concerned their rather high levels of education and professionalism, which were not considered representative for the average asthma or COPD patient. However, at the same time many interviewees indicated that these higher levels are necessary when patients are to be 'worthy' discussion partners of professionals.

> "I think you need good patient representatives. They need to be capable of holding their own during a discussion [with professionals]." (a COPD patient)

> "Patients who participate should have some knowledge of asthma, for example by reading information provided by the Asthma Foundation and by being member of the Asthma Foundation. They should have been ill for a number of years so that they can look beyond their own problems. In that case patient participation [in research agenda setting] could be useful for the Asthma Foundation." (a biomedical scientist)

During the final prioritization of this agenda the numbers of representatives from the three stakeholder groups differed as well, because of the non-response by several people approached.

Transparency of objectives, roles and structure

The objectives of the project as well as the respective roles of the NAF, the project team, and the different stakeholders to be involved, had been defined at the start of the process and were laid down in the initial project document and contract. Throughout the process all participants received information concerning the background, objectives, and structure of both the process and individual meetings or interviews. In addition, a summary of the consultation phase and its results was presented at the start of the integration meeting. When patients were involved, scientific or professional terms were translated into daily language.

Most interviewees mentioned that for them the process structure had been transparent. They indicated that the information provided was adequate and understandable and that objectives and roles were clear. Some points of criticism concerned the start of the consultation phase (especially for scientists and health care professionals) and the follow up of the project, i.e. the further specification and implementation of the research agenda:

> "I think at the start of the process many things were unclear. Probably the [NAF] office could not communicate very well what was going to happen. As from February 4 [the scientist consultation meeting], this improved and the process became transparent." (a biomedical scientist)

> "I knew that the meetings were part of the decision-making process. It was explained that there would be an important consultation round. However, I did not read the information from the Asthma Foundation on this in detail. Therefore it was very useful that the process structure was summarized again on April 2 [the integration meeting]." (a medical specialist/researcher)

> "For me the entire process was quite clear and transparent. […] I only do not know which steps are going to be taken now." (an asthma patient)

Early and direct involvement of patients

The consultation phase at the start of the project ensured the early and direct involvement of all relevant stakeholders. Also in the collaboration phase stakeholders were involved in a direct manner. Any interpretations and intermediary results were verified by sending participants reports after

all interviews and meetings with the request to comment on these reports. If necessary, results were amended.

Explicit incorporation of patients' inputs in discussions and decisions

Concerning their input into discussions and decision-making processes, patients stated on feedback forms and during evaluation interviews that they felt they had contributed to discussions and agenda-setting exercises.

> "I got good opportunities during the [integration] meeting. I definitely did not let me be pushed aside during the exercises. But I neither got the impression that people wanted to do that. We could consult each other and express whether we did or did not agree freely." (a COPD patient)

> "I could indicate what is important for me as a patient. In addition, I am glad that through my participation 'the patient' has had an input in the agenda setting." (an asthma patient)

This finding was confirmed by results from observation and tape analysis.

A remark by a few interviewees was that in principle there had not been a functional need to perform the integration of the priority lists in an interactive manner. However, they all acknowledged the surplus value of an interactive process with respect to the legitimacy of outcomes.

> "It was good to do it together, but the [integration] probably would have resulted in the same outcome without me, or when three other people had been absent. However, in this way it was much fairer and more broadly supported. Otherwise the feeling may have arisen that results could have been manipulated." (a biomedical scientist)

In general, the adequacy of the various procedures and the process structure was agreed upon by most participants and interviewees. Two patients indicated they had preferred the organization of an additional patient meeting just before the integration meeting in order to get acquainted with each other and to be able to discuss patients' priorities in preparation to the integration meeting.

Independent and unbiased management

The process management was in the hands of project team members who all were independent from both assigning bodies and the different stakeholders as well as unbiased with reference to asthma/COPD research.

Equality of participants

All participants interviewed had experienced to be treated equally in both the consultation meetings and the integration meeting. Procedures and discussions were generally regarded as fair, both within the consultation meetings and the integration meeting.

> "Fairness was ensured by the open discussions in the subgroups. […] In the subgroups everybody got a chance. To my opinion there was no top-down steering in a certain direction. In addition, the chairman ensured that people did not fall back in riding their hobbyhorses. Besides, there was a kind of social control within the group that was on the alert for these things. What was plenary discussed by the chairman later on was broadly supported by the group." (a biomedical scientist about the consultation meeting with scientists)

> "I experienced that I could freely speak and that discussions were fair. I felt that participants on April 2 were not opponents but partners what also was caused by the adequate classification in groups." (a COPD patient)

Concerning access to resources, all participants of each meeting were provided the same information beforehand and afterwards, as well as travelling expenses if requested.

Facilitation of mutual respect, openness, and constructive interaction

During the different meetings, atmospheres were open, respectful and collaborative. Participants indicated to have felt stimulated and free to express and explain themselves and to question each other.

> "The collaboration between participants was good and agreeable. There was mutual sympathy and people increasingly understood each other. There were different visions, but that is how it should be. I could express my thoughts without problems and they were listened to as well." (a medical specialist/researcher about the consultation meeting)

> "There was mutual respect and everybody listened to each others opinions. This certainly had a surplus value. This surplus value occurs because you are informed about the reasons why people have certain views on what is important and what is not." (a biomedical scientist about the integration meeting)

> "I did not have the feeling that everybody was talking from his or her own interest. I have experienced the integration meeting as a very pleasant meeting, also due to the skilful chairing. […] I experienced much interest and understanding towards the patient." (a COPD patient)

In this way constructive interaction between different stakeholders was facilitated. In general, the interviewees were quite satisfied about the management of the different meetings.

Regarding the fulfilment of process criteria we thus can conclude that the participation implemented can be considered quite effective in terms of legitimacy and rationality of the process. The overall process structure appeared to be quite adequate for accomplishing direct, early, and fair involvement of patients and their inputs in discussions and decision-making processes, while the process management seems to have been adequate in terms of ensuring equal treatment of participants, creating an open and respectful atmosphere, and facilitating constructive interaction. A less optimal aspect concerned the inadequacy of stakeholder representation during the integration meeting.

6.3.2 Evaluation of the outcomes

Consensus on resulting research agenda

The main (direct) outcome of the interactive research agenda-setting process consists of a shared 'societal' agenda for asthma/COPD research, comprising a wide range of concrete research topics, including biomedical, socio-cultural, health care, and policy research topics. This agenda resulted from the integration of the different research priority lists of the different stakeholder groups. During the integration meeting consensus between the stakeholders on integration and categorization of research topics was easily achieved. Also between the three subgroups during this meeting there was considerable consensus.

> "The process was enjoyable because everybody was taking part in it. Each group had more or less the same outcomes, indicating that these were broadly supported." (a biomedical scientist)

Afterwards, all participants interviewed indicated to agree with the results and to recognize their own views and priorities within the research agenda.

A second direct outcome of the project is the prioritized research agenda. This outcome was not based on consensus but was a combined result of 41 individual prioritizations. The 15 highest prioritized research topics concerned mainly biomedical and epidemiological research topics.

Reflection of patients' perspectives in research agenda

The societal research agenda adequately reflects the perspectives of all stakeholders involved. The specific contribution of patients, consisting of research priorities that were not mentioned by the other stakeholder groups during the consultation phase, is clearly traceable in the agenda.

In the prioritized research agenda, however, patients' actual influence is more difficult to trace. Although patients' highest priority during the consultation phase – research on the aetiology of asthma and COPD – was also prioritized during the final priority setting exercise, this does not prove patients' influence on the process outcomes since the same topic had been identified as a priority by most stakeholder groups during the consultation phase. Research on co-morbidity related to COPD was the only 'patient-specific' priority that was prioritized during the final prioritization.

Practicality of resulting research agenda

In general, the overall research agenda seems to be well substantiated and well translatable into further policy or action. All research topics included in the agenda had been founded on arguments provided during the consultation phase. In addition, both NAF managers interviewed indicated that they considered research agendas useful for further specification and implementation within their research policy, although they had expected different outcomes.

> "The expectation was that patients would prioritize a concrete need, such as improvement of health care. Instead the focus of patients was on biomedical research. [...] The results from the meetings with the different stakeholders, together with the integration of these results, are usable for further policy making." (one of the programme managers of the NAF)

Achieved learning on substantive, procedural and reflective matters

Concerning the indirect outcomes, both interviews and final prioritization results indicated that knowledge sharing had occurred. Several interviewees indicated to have learned something throughout the process. At a substantive level, some scientists and health care professionals stated to have learned about each others disciplines, both during the consultation phase and the collaboration phase.

> "I learned that hereditary aspects can be influenced from generation to generation. For example, the behaviour of the mother during

> pregnancy can influence the course of the disease. Most other research topics were rather familiar to me." (a health care professional)
>
> "I think the integration meeting was very informative and special. Prior to the meeting my perspective on biomedical science was rather limited. During the meeting I learned more about the types of research that are funded by the NAF." (a social scientist)

Patients indicated to have learned about disease and health care related matters from fellow patients as well as about professional matters from scientists and health care professionals.

> "Because within the meeting there was room to hear from other participants, I learned from the other patients. I have gained more insight in patients' perceptions of their environment as well as more in-depth knowledge about the disease." (a young asthma patient)
>
> "I noticed that scientists have strong views concerning research priority setting. This has strengthened my view on the importance of basic research." (a parent of an asthma patient)

At a procedural level, several participants (both patients and professionals) indicated to have learned something about interactive methods and processes, and their possible value.

> "A procedure as has taken place is interesting and I certainly have learned from it. The process of funnelling to a whole with different stakeholders with as much objectivity and as less directivity as possible is also a learning experience." (a medical specialist/researcher)
>
> "I learned a lot from the process. In particular that there was coherence between all activities that facilitates the alignment of all opinions and the eventual formulation of one list with priorities." (a COPD patient)

At a reflexive level, finally, almost half of the participants stated to have learned about their own and each others perspectives and priorities.

> "I learned that we [the scientists] do not always speak the same language. What is clear to me is not necessarily at the same time clear to someone else. How I think the world works is not necessarily the truth." (a biomedical scientist about the consultation meeting for scientists)
>
> "One mainly learns to speak each others language and to know each others motives. In addition, one learns to know each others hobbyhorses, as well as one's own." (a biomedical scientist about the integration meeting)

For example, some experts mentioned to have learned that patients are capable of thinking beyond individual problems and in (biomedical) long-term targets.

> "I learned about the visions of the patient group, namely that patients think about long-term solutions and not only about solutions for the short term." (a social scientist)

> "It was remarkable and good that patients had the insight that fundamental research is important as well. However, it was disappointing to me that aspects of health care were less present in both the patients' list and the list of health care professionals." (a health care professional)

> "The priority lists comprised several surprising aspects. An important aspect within the list of patient priorities was [the focus on] research on mechanisms of asthma and COPD. Although this was surprising, it is logical that patients regard this topic as important; the clarification of mechanisms of asthma and COPD could lead to better therapies and interventions. I also learned that patients judged co-morbidity an important topic. I have never before considered doing research on co-morbidity." (a biomedical scientist)

Other participants stated to be surprised about the consensus on various priorities between the different stakeholders. Also the NAF managers involved indicated to be surprised about patients' focus on biomedical research and about the consensus on priorities between the different stakeholders.

> "It was instructive to see that there was so much overlap between the priorities of the different groups. It was good to see this overlap and to notice that the components of the different lists mutually corresponded and that only the angles of the different groups differed." (a social scientist)

The occurrence of changes in thinking became also evident by the fact that there were some shifts in priority setting by stakeholders after the integration meeting. For example, while research on co-morbidity was not prioritized by health care professionals in the consultation phase, some professionals did prioritize this topic during the final priority setting in the collaboration phase (data not shown). As another example, research on smoking behaviour was not mentioned by biomedical scientists in the consultation phase but was prioritized as second highest by this stakeholder group in the collaboration phase (compare tables 6.2 and 6.6). Within this framework one biomedical scientist interviewed remarked that he noticed opinions about priorities growing closer to each other during the process:

> "I think the achieved outcome is a synthesis of all interests. During the process opinions have converged and have been adjusted along the way. I think this is indicative for the adequacy of the process outcome."

Also one of the patients involved explicitly mentioned his change of view:

> "I think I have filled in the matrix in a different way than I would have done without having attended the integration meeting. Through the meeting I underwent a learning process that also has changed my way of filling in the matrix." (a parent of an asthma patient)

There thus had been some implicit and explicit mutual learning between the several participants involved.

With respect to the process outcome and the extent of knowledge sharing we thus can conclude that our participation exercise seems rather effective as well. Patients, just as the professional participants, have clearly contributed to the main process outcome: the societal asthma/COPD health research agenda. However, with respect to the further prioritization of this agenda, patients' influence on the outcome was less clearly traceable. Finally, the achievement of some knowledge sharing suggests that objectives of increase in social capital and human capital may be achieved as well.

6.4 Discussion

The results of our evaluation indicate that the proposed strategy for patient participation in research agenda setting realizing can be regarded as appropriate for realizing effective participation in terms of achieving its main objectives. However, the participation exercise realized showed some suboptimal aspects as well. One suboptimal aspect was the under-representation of some stakeholder groups during the last two phases, as a result of late excuses and non-response. However, we can state that this suboptimal stakeholder representation did not affect the legitimacy of the overall process nor the quality of the outcome. The overall representation of the different stakeholders during the consultation phase was adequate and all consultation results were incorporated unchanged into the end result.

Another suboptimal aspect of the participation exercise concerns the limited reflection of patients' original perspectives in the final prioritized research agenda. This aspect reflects a more serious problem. Patients' highest priorities during the final priority setting exercise (see table 6.6) deviated from the priorities of patients as indicated in the questionnaire (table 5.3). In the final prioritization patients hardly

prioritized research on co-morbidity or on drug interaction (polypharmacy) or side effects, which were high priorities of patients during the consultation phase. Only co-morbidity related to COPD was prioritized by some patients and finally included in the prioritized agenda.

Several explanations can be given for this change in patients' priorities. Firstly, the 15 patients participating in the final priority setting may not be representative for the entire patient community that had been consulted before, although the stratified analysis of the questionnaire indicated that neither demographic nor disease characteristics significantly influenced research priorities. Possibly, a higher level of proto-professionalism has resulted in different research priorities, for example through the loss of original perspectives. Secondly, research on the aetiology of asthma and COPD involved one topic within patients' questionnaire but covered five topics that patients could prioritize in the final priority setting exercise. Patients' prioritization of aetiological research topics may have pushed aside other priorities. Thirdly, patients' priorities may have changed after 'learning' from the professional participants. Patients (implicitly) might have adopted research priorities from professionals or changed their own views concerning research priorities. For example, during the integration meeting some professionals stressed (although not agreed by all professionals!) that research on medication is the territory of the pharmaceutical industry and should not be included in a research agenda for the NAF and ZonMw. Likewise, topics related to the reduction of costs were considered policy topics rather than research topics by most professionals. Possibly patients have internalized opinions like these and have changed their own priorities. One of the patients involved explicitly mentioned this change of view:

> "I think I have filled in the matrix in a different way than I would have done without having attended the integration meeting. Through the meeting I underwent a learning process that also has changed my way of filling in the matrix." (a parent of an asthma patient)

It is here that we face two additional complications. Firstly, changes of thinking among patients could be caused by mutual learning as well as by the overruling of patients by professionals. Distinguishing mutual learning from overruling in this kind of processes is very difficult, if possible. Moreover, even careful guiding of interactive processes by experienced process managers may not prevent some overruling of 'lay people' by experts. Secondly, mutual learning may facilitate (future) participation and integration processes but may also result in a loss of

original perspectives. As we have argued previously, the loss of original perspectives implies the loss of possibly valuable patients' experiential knowledge (Caron-Flinterman et al., 2005) that could contribute to the quality of decision-making. At the same time it seriously complicates the assessment of the integration of patients' perspectives in final decisions, which is one of our criteria for effective participation. This dilemma calls for further research.

Finally, we want to reflect on an additional criterion that was excluded from our evaluation framework: the actual impact of the participatory results on further policy and action. This criterion is considered very important by several scholars dealing with public or stakeholder participation (e.g. Guston, 1999; Rowe and Frewer, 2000). In addition, several participants that were interviewed in our evaluation study indicated that they wanted to postpone their overall opinion on the effectiveness of the agenda-setting process until the actual impact of the agenda on eventual research programmes would be clear. Indeed, if the resulting research agenda is actually translated into concrete research programmes by the NAF, ZonMw, or other research financiers, and eventually leads to the execution of alternative research projects, patients can be said to have contributed to the quality and relevance of research. In that case, the interactive research agenda-setting project can be considered effective with respect to the long term as well.

However, the long-term impact of the agenda is only indirectly influenced by the participation methodology used. It strongly depends on the commitment of the NAF and ZonMw management to both the agenda-setting process and its outcomes. In turn, this commitment depends on the rationality and legitimacy of the agenda-setting process, the roles of the NAF and ZonMw management in the design and execution of the process, the congruency of the resulting agenda with expectations and existing policy lines, and their (felt) dependency on the support of stakeholder groups. Other factors that contribute to the eventual adoption of the agenda are the translation and specification procedures followed, the room for manoeuvring within existing policy lines, the success of lobbying activities, the receptiveness and influence of other relevant actors in the field, the willingness of individual researchers to submit alternative research proposals, etc. We therefore abstained from evaluating the longer-term impact of the project results on further policy and action. However, the fact that the NAF management is currently using the 'societal agenda' concerning asthma/COPD research as a guideline for the next research

programming implies a promise towards some long-term impact of the participation exercise.

6.5 Concluding remarks

Our patient participation strategy, including an elaborated consultation phase and a collaboration phase, and focussing on the facilitation of mutual learning and constructive interaction, can be considered an adequate strategy for implementing effective patient participation in the agenda setting of asthma/COPD research. By making patients priorities explicit and subsequently integrating these priorities in the resulting research agenda in a deliberative way, it overcomes the main inadequacies of current strategies for patient participation that only involve consultation or collaboration. It realized the main objectives of patient participation in research agenda setting as had been identified before: it provided a legitimate and rational process that resulted in the actual influence of patients on the main process outcome: a 'societal research agenda', which was generally considered quite substantial and useful and which serves as a guideline for research programming by the NAF management. In addition, as an indirect outcome, some knowledge sharing between participants had taken place, resulting in changes of thinking that might facilitate possible future interactive research agenda-setting processes.

One could reasonably assume that a similar strategy may be appropriate for the implementation of effective patient participation in other research agenda-setting contexts as well. For example, although the agenda-setting process concerned the entire breadth of health research on asthma and COPD, we argue that the strategy is quite well applicable within the specific context of biomedical research as well. After all, a substantial part of the research agenda concerned biomedical research and patients proved to be capable of reflecting on, and prioritizing biomedical research topics (see chapter 5).

Further research should focus on the actual longer term impact of the participation exercise as well as on finding a balance between striving for mutual learning and requiring the adequate reflection of patients' inputs in final results.

7

CONCLUSIONS

This study aimed to analyse the current situation concerning patient participation in decision-making on biomedical research and to contribute to the development and implementation of a strategy that could realize patient participation in a more structural and effective way. The main research question addressed in this dissertation is:

> *"To what extent is effective patient participation in biomedical research decision-making possible?"*

In order to answer this question, I formulated three sub-questions that determined the outline of the study:

> *I. What causes the apparent limited participation of patients in decision-making on biomedical research agendas?*
>
> *II. What strategy could be devised to include them more actively and effectively in biomedical research decision-making processes?*
>
> *III. What can we learn from the practical implementation of this strategy, in particular in terms of effectiveness?*

The study thus consisted of three different research parts: a more descriptive-analytical part, a more conceptual part, and a social-experimental part that followed each other in a more or less chronological way.

In order to answer the main research question, twelve detailed research questions were formulated during the course of the study (see section 1.3.2) and subsequently addressed in the chapters 2 to 6. In the sections below the main findings and conclusions of the study are summarized and discussed. Based on these findings and conclusions the three sub-questions will be answered. Subsequently, the main conclusions and their contribution to the scientific and societal objectives of the study

will be discussed. In the last section, I will give some suggestions for further research.

7.1 The current situation

The first sub-question focused on the current situation concerning initiatives of, strategies for, as well as obstacles to, patient participation in decision-making on biomedical research (research questions 1 to 4). Data had been collected by means of interviews and desk studies.

Firstly, the findings presented in chapters 2 and 3 indicate that patients are still *rarely involved* in decision-making on biomedical research and that if they are involved, it is on an ad hoc basis. Subsequently, it was found that in the examples identified in the Netherlands, patient participation in decision-making on biomedical research was mainly achieved by following one out of *three strategies:* (1) the successful lobbying of patient organizations for certain types of research, (2) the ad hoc use of patients' ideas and demands as research questions, topics, or priorities via intermediaries, and (3) the membership of patient representatives in existing decision-making research committees or councils. Although this part of the study focused on the situation in the Netherlands, literature research and informal communication with informants involved with patient participation in other countries have indicated that these findings apply broadly.

The three strategies identified were analysed in terms of 'levels' of participation. For this purpose, Sherry Arnstein's participation ladder (1969) (see figures 1.1 and table 1.1) was used as a framework. The first two strategies identified were labelled as forms of 'consultation', a minimal level of participation in Arnstein's participation ladder. Within these strategies patients or patients' organizations either push forward their views or are consulted on their views by intermediaries, but professionals eventually decide about the use of these views. Patients thus are not directly involved in the decision-making process itself. In addition, there is no guarantee that patients actually influence decision-making on research policy or projects. The fact that these forms of consultation usually occur in a rather ad hoc and non-structural manner further limits patients' potential influence.

The third strategy identified reflects a higher participation level of 'placation' or 'partnership', depending on the actual power sharing

between patients and professionals.[62] However, interviewees stressed that in practice this kind of participation seldom reflects real 'partnership'. Usually only one or two patient members have to face a majority of (different) professional members, which easily results in placation instead of partnership. In addition, since the actual influence of patients on outcomes strongly depends on their proto-professionalism and empowerment, their representativeness is limited. Moreover, decision-making processes within committees or councils are far from transparent since they comprise lobbying, private negotiation, and strategic behaviour. As a result, even patient members of committees who felt taken seriously and heard could not mention any concrete example of their actual influence on overall decision-making.

Subsequently the appropriateness of the three strategies for realizing effective participation was estimated, in terms of ensuring the achievement of generally acknowledged objectives of stakeholder participation. As a definition it was stated that effective patient participation should accomplish a form of partnership, involving the direct and early involvement of patients in fair decision-making processes that include negotiation, deliberation and power-sharing as well as the structural integration of participants' knowledge in, and thus their actual influence on, process outcomes. Concerning the three identified strategies, it was concluded that, although individual implementations may have resulted in changed research agendas, none of them can be considered as realizing effective participation. The first two strategies end in some actual influence of patients on biomedical research agendas but only in an ad hoc manner without realizing any partnership, while the third strategy structurally involves patients in decision-making but cannot warrant their actual influence on the outcomes.

Since in other research fields effective participation has been successfully implemented, it was suggested that some *obstacles* might hamper effective patient participation in decision-making on biomedical research. Interviews with relevant actors indicated that obstacles for effective patient participation are related to limited resources, such as time and money, to structural and cultural characteristics of the current biomedical research decision-making network, and to characteristics of the patient community that is to be involved in this network. These obstacles together reflect a kind of resistance of the biomedical research decision-

[62] In the literature on patient participation this kind of strategy is usually termed 'collaboration', which can refer to either placation or partnership.

making network and its stabilized ways of thinking and acting (its 'regime') towards change in terms of structurally involving patients in decision-making. Obstacles attributed to the characteristics of patients mainly refer to patients' limited capability to contribute anything relevant to decision-making on biomedical research, because of their limited knowledge, interests, and competencies. Since this category of obstacles implicitly refers to the incongruence of patients' characteristics with the current regime of the biomedical decision-making network, it could be regarded as reflecting network characteristics as well.

7.2 Patients' capabilities

One of the obstacles for effective patient participation identified was the general adhered presupposition that patients are incapable of adequately participating in decision-making on biomedical research, because of their lack of relevant knowledge, their lack of objectivity, and their lack of interest in long term, biomedical research targets. Since this presupposition contradicts the important substantive argument for patient participation which builds on the potential expert-role of patients, it justifies more in-depth study. Therefore the tenability of this presupposition – and thus simultaneously the tenability of the substantive argument for patient participation – was assessed by investigating the capability of patients to contribute something relevant to decision-making on biomedical research in practice.

Firstly, research questions 5 and 6 addressed the potential contribution patients could make to decision-making processes regarding biomedical research. In answer to these questions, in chapter 3 it was suggested that patients' potential contribution is primarily related to their *'experiential knowledge'*. However, it appeared to be difficult to make a conclusive judgement on whether patients' experiential knowledge can be considered valid, since this judgement depends on the (epistemological) paradigm adhered to. Therefore, a pragmatist approach was followed by estimating the validity of patients' experiential knowledge in terms of its practical value by investigating concrete examples of the application of this knowledge – expressed in ideas, demands, or judgements – in biomedical research decision-making.

Although extensive literature research and interviews resulted in the identification of only few concrete examples, these examples indeed

suggest that patients' experiential knowledge has potential value for decision-making regarding biomedical research. In these examples patients' experiential knowledge had been translated into explicit demands or ideas that formed a direct input to biomedical research processes as new research priorities, topics, or hypotheses.

Secondly, research questions 9 to 11 addressed patients' actual *capability* of participating adequately in (biomedical) research agenda-setting processes. These research questions were addressed in chapter 5, which described the consultation of asthma or COPD patients (members of the NAF; the Netherlands Asthma Foundation) on their health research priorities within the context of an interactive asthma/COPD health research agenda-setting process (see next section). The patients were successively consulted by means of exploratory focus groups, a feedback meeting, and a questionnaire.

The results of the questionnaire indicated that NAF members prioritized biomedical and epidemiological research topics higher than topics concerning health services research or socio-cultural research. In addition, the focus group discussions indicated that NAF members were able to substantiate their perspectives on priorities. Although this study was restricted to the consultation of NAF members only, an additional questionnaire among asthma/COPD patients who were not member of the NAF demonstrated that there was no difference in research priorities between members and non-members.

The results of this consultation contradict the presupposition of many people (both experts and patients) that patients are not capable of participating adequately in biomedical research agenda setting. Firstly, asthma/COPD patients seemed to obtain enough knowledge and information to formulate and prioritize health research topics covering the entire health research field, including biomedical research. Secondly, they appeared to be able to think in biomedical, long-term targets and in favour of future generations and did not only focus on personal health care and social problems. Thirdly, asthma/COPD patients were capable of introducing some new biomedical research topics that were neither covered by current NAF research agendas nor by the research priorities of other stakeholder groups: co-morbidity, side effects of medication, and mutual interaction of medication. Although obtained in the framework of health research agenda setting, these findings indicate that asthma/COPD patients are very well capable of participation in biomedical research agenda setting as well, since they appeared to be able to suggest, reflect on,

and prioritize biomedical research topics. One could reasonably assume that other patient groups will be able to do the same.

These findings thus refute the notions that participating in decision-making on biomedical research requires highly specialized knowledge and that patients could not contribute to this decision-making. Instead, it could be concluded that patients, based on their experiential knowledge, have something new to contribute, which substantiates the substantive argument for patient participation in biomedical agenda setting.

7.3 A transdisciplinary strategy

Although the existence of different participation initiatives indicates that actors already experiment with patient participation in decision-making on biomedical research, its structural and effective implementation requires a change of the current decision-making network and its regime, which could be considered a 'transition'. Transition management may offer a way to breach the resistance of the biomedical research decision-making network and to realize a change of the network towards effective patient participation.

In chapter 2 it was argued that such a transition could be induced by the portrayal of successful examples of effective patient participation in decision-making on biomedical research. Therefore, the research questions 7 and 8 focused on what the design of a suitable strategy for effective patient participation could look like. Based on the findings of the previous chapters, on literature research, and on earlier experiences of the Athena Institute, a new patient participation strategy (or methodology; see note 40) was formulated in chapter 4.

The participation strategy proposed was based on the Interactive Learning and Action (ILA) approach, which was developed by members of the Athena Institute (department of Biology and Society) for the gearing of agricultural innovation processes towards the needs and interests of small-scale farmers in developing countries (Broerse and Bunders, 2000). This approach has proven to be successful in realizing stakeholder participation in decision-making on science and technology. Since effective patient participation in decision-making on biomedical research implies, among others, involving patients in an early, direct, and fair way while ensuring their influence on process outcomes, the participation strategy

included separate *consultation and collaboration*[63] phases. In order to utilize the full potential of patients' knowledge, the strategy had a *transdisciplinary*[64] character, explicitly and deliberately integrating patients' experiential knowledge with the knowledge of biomedical researchers and other professionals.

The new patient participation strategy is a phased, transdisciplinary strategy that aims to facilitate equality, constructive interaction, mutual learning, and knowledge integration between patients, biomedical researchers, and other relevant actors. It consists of six phases:

1. *Preparation and initiation* This phase comprises preliminary research that aims to identify relevant actors and to assess current patterns of thinking and acting within the biomedical field of interest, by means of desk studies and interviews. In addition, the objectives of the process, the methods to be used, and the roles of the participants are specified, while a process management team is established in order to guide the process.
2. *Consultation* This phase aims to gather information about the perspectives and views of actors involved. For this purpose a variety of techniques can be used, such as interviews, brainstorming sessions, discussion meetings, focus groups, and questionnaires. Different stakeholder groups may demand different consultation techniques.
3. *Collaboration* Subsequently, in the third phase the different actors are brought together in interactive settings in order to discuss these perspectives and views and to integrate them into one shared construction. Interactive settings should be carefully guided in order to facilitate and stimulate mutual openness and respect, constructive interaction, and mutual learning.
4. *Prioritization* In this phase participants identify priorities. In order to prevent undue mutual influencing such as the overruling of

[63] The term 'collaboration' is usually used to indicate a way of participation that implies the structural involvement of patients in formal decision-making structures such as committees or boards. 'Collaboration' can thus refer to both Arnstein's participation levels of 'placation' and 'partnership'. In practice it is often very difficult to distinguish placation from true partnership because the actual influence of patients in decision-making within mixed committees or boards is hard to assess.

[64] The term 'transdisciplinary' refers to the integration of both scientific and non-scientific knowledge in problem-solving or decision-making processes in search for a more integral and 'socially robust' knowledge.

patients by the views and priorities of experts and professionals, prioritization should preferably occur on an individual basis.

5. *Specification* The fifth phase comprises the translation and specification of priorities identified into a concrete plan of action, comprising for example research programmes, projects, or policy actions. Although this phase is often in hands of the 'assigning body' that has commissioned the patient participation initiative, the involvement of stakeholders can ensure that the resulting plan of action reflect their priorities.
6. *Implementation* The final phase comprises the implementation of the established plan of action. This phase usually is in the hands of the 'assigning body' as well. Ideally also in this phase stakeholder groups are actively involved.

Additional conditions identified for the successful implementation of such a transdisciplinary strategy comprise conditions for the social setting as well as for the members of the process management team. For example, the social setting should be characterized by mutual equality and coalition building between participants in an open and trustful atmosphere that stimulates rational discourse, feedback, mutual learning, and the achievement of shared constructions. In addition there should be clarity about process objectives and mutual expectations as well as some room for manoeuvring and negotiation. The process management team should possess both scientific and communication/organizational skills and should be able to generate the appropriate social setting and to guide constructive and transdisciplinary interaction between participants in an open, respectful, and creative way.

7.4 Implementation and evaluation of the strategy

In a social experiment, described in chapters 5 and 6, the proposed transdisciplinary strategy for effective patient participation in decision-making on biomedical research agendas was *implemented* in a concrete setting and *evaluated* in terms of accomplishing effective participation. This part of the study addresses research question 12. The implementation took place in the context of a broader participative health research agenda-setting project that was commissioned by the Netherlands Asthma Foundation (NAF) and co-financed by the Netherlands Organization for Health Research and Development (ZonMw).

The overall participative research agenda-setting project consisted of the first four phases of the transdisciplinary strategy described above. In a preparation and initiation phase the asthma/COPD research field and its decision-making patterns were assessed. In addition, the project team and process design were established and the process objectives were defined.

In a consultation phase the three main stakeholders of asthma/COPD research – researchers, health care professionals, and patients – were consulted on their views concerning asthma/COPD research priorities from the perspective of their specific background and expertise. To this end different consultation methods were used. As had been argued in chapter 5, the combination of focus groups and a questionnaire constituted an appropriate methodology for investigating patients' priorities on research. The questionnaire was a suitable tool for explicitly consulting a representative group of patients on their research priorities, without becoming obscured by group effects; the input from focus groups was indispensable for getting a proper design of the questionnaire as well as for gaining insight in underlying arguments and perspectives. The other stakeholder groups were consulted by means of discussion meetings and interviews.

Subsequently, in a collaboration phase all research priorities of the different stakeholders – including many (expert) members of current research committees – were discussed and integrated into one 'societal' research agenda during an integration meeting. In a final prioritization phase participants were individually asked to prioritize this agenda using a prioritization matrix.

In chapter 6 the appropriateness of the implemented participation strategy was evaluated in terms of accomplishing effective patient participation. This evaluation took place with respect to various process and outcome criteria, which were formulated on the basis of participation objectives considered relevant within the context of the interactive asthma/COPD research agenda setting process: (1) the legitimacy and rationality of the agenda-setting process, (2) the quality of the resulting agenda in terms of usefulness and adequate reflection of stakeholders' perspectives, and (3) the occurrence of mutual learning between stakeholders involved.

The evaluation results indicated that the transdisciplinary strategy used for the implementation of patient participation in research agenda setting was appropriate for realizing effective participation. The strategy gave rise to a legitimate and rational process that resulted in the actual influence of patients on a societal research agenda, which was generally

considered useful and which serves as a guideline for further research programming. Patients introduced three new biomedical research topics into the research agenda that were not mentioned by the other stakeholders: research on co-morbidity, on side effects of medication, and on drug interaction. These topics can be considered as originating from patients' experiential knowledge. In addition, as an indirect outcome, mutual learning between participants had taken place, resulting in changes of thinking that might facilitate possible future interactive research agenda-setting processes. Although the agenda-setting process focused on health research, it can be assumed that patient participation in biomedical research agenda setting can be successfully designed in a similar way.

Sub-optimal aspects of the participation exercise were (1) the somewhat low and unequal representation of the different stakeholder groups during the last two phases, as a result of late excuses and non-response among all stakeholder groups, and (2) the relatively poor reflection of patients' influence in the final prioritized research agenda. Probably, the most obvious explanation for the latter issue is that patients participating in the final priority setting were not (or no longer after mutual learning) representative for the patient community that had been consulted before and did not (or no longer) consider the earlier identified highest priorities of patients as most urgent.

7.5 Overall conclusion

Based on all the findings summarized in this chapter, the three sub-questions can be answered as follows.

The results of the descriptive-analytical part of the study lead to the conclusion that the 'apparent limited participation of patients', which was assumed in the introduction, is not the result of inadequate documentation of patient participation initiatives but corresponds with the actual practice of decision-making concerning biomedical research agendas. The study showed that patients are currently involved in decision-making concerning biomedical research only on a limited scale and often in an ineffective way (in terms of involving patients early and directly in fair decision-making processes that ensure their influence on the decision outcome). Secondly, this limited patient participation can be considered as being caused by the resistance of the current biomedical research decision-making network, and its regime, towards change.

The findings of the designing part of the study suggest that a transdisciplinary strategy, including both an explicit consultation and a collaboration phase, and enhancing constructive interaction and knowledge integration, could be an appropriate patient participation strategy. The results of the social-experimental part of the study confirm that the proposed strategy indeed provides a suitable strategy for realizing effective patient participation in decision-making on biomedical research. In addition, both the analysis of earlier cases of patient participation and the social experiment indicate that patients are potentially capable of identifying, valuing, and prioritizing biomedical research topics, and that they have something new to contribute to biomedical agenda setting.

Concerning the *main research question*, I thus can conclude that although effective patient participation in biomedical research decision-making is not common practice, its implementation is possible when following a carefully developed transdisciplinary strategy that includes explicit phases of consultation and collaboration (in terms of partnership) and that stimulates constructive interaction and knowledge integration between actors involved.

The study described in this dissertation contributed to scientific knowledge production on strategies for effective patient participation within the biomedical sector. It has resulted in increased insight in the value of patients' experiential knowledge for biomedical research, in obstacles that hamper effective patient participation in decision-making on biomedical research, and in conditions and procedural elements that stimulate and facilitate constructive interaction and knowledge integration between patients and professionals. Since in other scientific fields comparable obstacles and opportunities are faced, many of the insights obtained probably could be generalized to other contexts of stakeholder participation.

On a societal level, the implementation of the developed strategy for effective patient participation within the context of an asthma/COPD health research agenda-setting process has contributed to the construction of a useful and broadly supported research agenda for asthma and COPD research. The developed strategy proved to be appropriate for the implementation of effective patient participation in decision-making on health research as well as on biomedical research. When further optimized and adapted it may form the basis for a broader applicable stakeholder participation strategy as well.

Finally, by providing both scientific insights on, and a successful societal application of, patient participation in biomedical research

decision-making, the study described in this dissertation might contribute to the induction of a transition of the biomedical decision-making network towards the structural inclusion of patients. It may dispel the largely held presupposition that patients are not capable of participating in decision-making on biomedical research in an adequate way and convince an increasing number of actors from the network about the feasibility and the added value of patient participation in decision-making. Similar changes in thinking were already observed in the social experiment concerning patient participation in asthma/COPD health agenda setting. Most participants of the agenda-setting process were quite enthusiastic about the interactive strategy followed and its outcomes. In addition, knowledge production on strategies for effective participation and their (pre)conditions will smooth future participation initiatives, also within other contexts.

Further signs of a transition towards a more structural involvement of patients in decision-making on research may soon become visible. Since the publication and distribution of the research report on the interactive research agenda-setting project by the NAF, other charity funds and patients' organizations are exploring possibilities for initiating similar processes.

7.6 Discussion

Concerning the first part of the study a point of discussion concerns the tenability of the conclusion that patient participation in decision-making on biomedical research occurs rarely and usually in an ineffective way. This conclusion was based on the facts that only a limited amount of examples could be identified and that these examples hardly reflected effective participation. Consulting additional patients' organizations or funding agencies might have resulted in some additional examples that might comprise other participation strategies. Nevertheless, the quite large number and variety of interviews held and the extensiveness of the literature research conducted to some extent ensured the validity of the outcomes. In addition, the many obstacles for effective patient participation identified further strengthen these findings.

Of all the obstacles identified, only the one concerning patients' apparent limited capability to participate adequately in, and to contribute to, biomedical research decision-making was explicitly addressed in this study. On the one hand, this obstacle was considered essential since it

questions one of the central arguments in favour of patient participation, which is based on the potential substantive contribution patients could make to biomedical research decision-making. Thereby, it strongly determines people's belief or disbelief in the potential success and value of patient participation efforts, which in turn determines whether people are willing to experiment with it. On the other hand, this obstacle could be refuted rather easily when it can be demonstrated that patients are capable of participating adequately in biomedical research agenda setting processes in practice. For example, the evaluation results of the participative research agenda setting process conducted and described in this study showed that several expert participants admitted to have adjusted their opinion on patients' capabilities based on the results of the patient consultation step.

Concerning the last part of the study, a critical remark should be made. The design and management as well as the evaluation of the participation initiative were in the hands of the project team involved. The evaluation thus can be considered a kind of self-evaluation, which strongly bears the danger of bias. This bias was minimized by: (1) ensuring that those who conducted the evaluation study were not the same as those who designed and managed the process, (2) involving all participants in the evaluation process in an open way, (3) applying triangulation of evaluation methods and member checks, and (4) presenting rich data.

Another discussion point concerns the sub-optimal elements of the designed transdisciplinary patient participation strategy that need improvement. Firstly, the representation of stakeholder groups in the collaboration and prioritization phases could be improved, for example by inviting more and equal numbers of stakeholder representatives. In this respect, it should be discussed whether equal numbers of all stakeholders need to be involved in the integration meeting or equal numbers of patients and experts. However, since it cannot be prevented that people call off attending a meeting at the last moment, the adequate representation of stakeholder groups can never be guaranteed in advance.

A second aspect that demands optimization is the explicit and traceable inclusion of patients' priorities in the prioritized research agenda. However, as has already been discussed in chapter 6, this optimization faces a dilemma. Involving a more representative group of patients in the final priority setting exercise might result in a better and more visible inclusion of patients' original research priorities in the final prioritization, but at the same time reduce the effects of mutual learning, something that has been considered an important condition for adequate participation processes. This dilemma refers to a more general dilemma in effective

participation processes: the two evaluation criteria mutual learning and representativeness seem to exclude each other to some extent. Mutual learning may result in a loss of original perspectives (and thus of potentially valuable patients' experiential knowledge) and complicates the assessment of patients' influence on final outcomes. When designing and/or evaluating a patient participation initiative, one should seek a balance in meeting both criteria.

Another optimization of the strategy could involve the insertion of additional meetings, such as a second patient meeting to discuss the questionnaire outcomes, (extra) meetings with socio-cultural scientists and health care professionals in order to discuss research priorities of these stakeholder groups more thoroughly, and an additional feedback meeting after the integration meeting or the prioritization exercise, which could optimize the substantiation of stakeholder priorities, the mutual learning in and between stakeholder groups, as well as the social support of the prioritized agenda even more. However, since these extra meetings will considerably increase the total costs, it is questionable whether this can be regarded as cost-effective.

A criterion that was excluded from our evaluation framework, but what is considered important by scholars in the field of stakeholder participation as well as by participants in the social experiment, concerned the actual impact of participatory results on further policy and action. Indeed, when the resulting asthma/COPD health research agenda is actually translated into concrete research programmes by the NAF, ZonMw, or other research financiers, and eventually leads to the execution of research projects that reflect the societal research agenda, patients can be said to have contributed to the quality and relevance of research. However, since this final impact of the developed societal agenda depends on a number of factors that lie beyond the scope of this study, the evaluation of the participation exercise has been restricted to the short-term outcomes of the project. It would therefore certainly be very interesting to assess the final impact of the societal asthma/COPD research agenda on the concrete research practice two or three years from now. The fact that the societal research agenda currently serves as a guideline for research programming by the NAF indicates that this impact may become visible.

A final remark concerns some stakeholder groups that have not been involved in the interactive agenda-setting project. One of those is the group of supporters of the NAF. Since they provide the major part of the funds that can be spend on research, and thus are relevant stakeholders,

they could be ascribed the right to participate in decision-making on research agendas as well. In addition, since ZonMw is also a sponsor and user of the agenda-setting project, it could be argued that the government and even the public at large should have participated in the agenda-setting process. In the framework of this study these stakeholder groups are excluded from participation since the NAF decided to involve only the three 'core' (expert) stakeholder groups in the agenda-setting process and possibly to involve other relevant stakeholders at a later stage.

7.7 Further research

I would like to close this dissertation by presenting some ideas for further research. Firstly, further research should comprise a follow-up study after three years or so in order to measure the impact of this social experiment on the research policy and decision culture of the NAF and ZonMw as well as on eventual asthma/COPD research projects executed. This research thus would involve the evaluation of the implementation phase of the participation strategy. It may lead to the identification and analysis of variables that could stimulate successful implementation of participation outcomes and thus could increase the effectiveness of the participation initiative in terms of actual impact.

At the same time, additional research could comprise the more thoroughly investigation of the social experiment described in this dissertation and its outcomes. For example the mutual interaction between the different stakeholder groups during the integration meeting could be investigated in more detail by conducting tape-analysis. Likewise it would be interesting to inquire the more specific role of patients' experiential knowledge on criteria they use for priority setting. These aspects may enrich insights in knowledge integration processes and conditions for their optimization.

In addition, further research could address the optimization of the participation strategy proposed and implemented in this study. As described above, this optimization may concern the representativeness of stakeholders involved in the collaboration and prioritization phases, the more explicit traceability of patients' influence on final outcomes, and the efficiency of the strategy. It would be interesting to investigate whether it is possible to obtain similar levels of effectiveness with less time and

reduced costs. For this purpose slightly adapted versions of the strategy should be repeatedly applied.

Other research could focus on the applicability of the formulated participation strategy within alternative contexts, concerning other patient communities or even other scientific fields. In comparison with other patient communities, the community of asthma/COPD patients is quite mobile, while the community of NAF members in particular is rather active and well-organized, as well as relatively well informed about developments within the research field (amongst others by the patient magazine 'Contrastma'). Other patient communities may demand additional efforts and facilities, for instance when being invited to attend group meetings. The application of the participation strategy in other scientific fields may require further adaptations.

Finally, additional research could address a more structural implementation, and eventually the sustainable institutionalization, of patient participation in established biomedical decision-making structures. For this purpose several steps could be undertaken. For example, managers of research funding agencies and other central actors in decision-making on biomedical research agendas could be made aware of and trained in the execution of participatory agenda setting processes. In addition, research could focus on making the strategy more efficient, in particular for follow-up agenda-setting processes. Furthermore, the training of biomedical students in transdisciplinary and participatory approaches could gradually change the strong disciplinary biomedical culture towards more openness and flexibility with respect to the participation of patients and their knowledge in decision-making processes.

SAMENVATTING

De laatste decennia vindt er binnen de wetenschap een toenemende democratisering van besluitvorming plaats. Enerzijds wordt op nationaal en internationaal niveau het grote publiek steeds vaker betrokken bij besluitvorming over controversiële wetenschappelijke ontwikkelingen, zoals binnen de biotechnologie en de genetische geneeskunde. Door het organiseren van publieke debatten of consensusconferenties probeert men draagvlak te creëren voor beleid en eventuele maatschappelijke weerstand te voorkomen. Anderzijds worden binnen bepaalde (meestal toegepaste) wetenschapsgebieden in toenemende mate relevante maatschappelijke actoren – zoals eindgebruikers, financiers of beleidsmakers – betrokken bij besluitvorming over de inhoud van onderzoeksprogramma's, -projecten of zelfs over oplossingsrichtingen. Behalve dat deze maatschappelijke actoren belangrijke *stakeholders* van dergelijk wetenschappelijk onderzoek zijn, zijn ze ook *experts* omdat ze een bepaalde vorm van contextuele kennis hebben die onmisbaar is voor het succesvol vormgeven van wetenschappelijke processen. Deze kennis wordt wel locale kennis, lekenkennis of ervaringskennis genoemd.

Binnen wetenschapsgebieden als milieuwetenschappen, planologie, agrarische wetenschappen, ontwikkelingsstudies en vele vormen van gezondheidsonderzoek wordt in de literatuur steeds vaker gerefereerd naar dergelijke vormen van 'stakeholderparticipatie' in besluitvorming over onderzoek. Echter, de biomedische wetenschap lijkt op het eerste gezicht gevrijwaard van stakeholderparticipatie in besluitvorming. Dit is op zijn minst opmerkelijk aangezien de biomedische wetenschap grote maatschappelijke belangen herbergt. Immers, het stelt zich ten doel bij te dragen aan de gezondheid van de mens, en daarmee aan de kwaliteit van leven. Bovendien wordt er, internationaal gezien, zeer veel geld in geïnvesteerd. Het doel van dit proefschrift is dit fenomeen nader te onderzoeken.

Biomedisch onderzoek is een 'technische' vorm van gezondheidsonderzoek die zich richt op het verkrijgen van inzicht in de

fysische mechanismen en processen die ten grondslag liggen aan gezondheid en ziekte. Daarmee vormt biomedisch onderzoek een belangrijke wetenschappelijke basis van onze westerse geneeskunde. Subdisciplines binnen het biomedisch onderzoek zijn immunologie, pathologie, neurologie, genetica, etc. Een belangrijke stakeholdergroep wordt gevormd door de patiënten, die de belangrijkste doelgroep en tevens eindgebruikersgroep van biomedisch onderzoek vormen. Bovendien zijn patiënten te beschouwen als (niet-gecertificeerde) experts omdat zij op basis van hun ervaringen met hun ziekte een vorm van ervaringskennis opbouwen die complementair is aan professionele, biomedische kennis en mogelijk een belangrijke bijdrage biedt aan de sturing van biomedisch onderzoek. Op grond van dit stakeholder- en expert-zijn van patiënten pleiten verschillende normatieve (ethische en politieke) en inhoudelijke argumenten ervoor hen actief te betrekken bij besluitvorming rondom biomedische onderzoeksagenda's. Patiëntenparticipatie in besluitvorming rondom biomedisch onderzoek kan bijvoorbeeld bijdragen aan de legitimiteit en de rationaliteit van besluitvormingsprocessen, aan de kwaliteit van genomen besluiten (bijvoorbeeld in termen van maatschappelijke relevantie van onderzoeksprojecten of –programma's) en aan een verhoogde maatschappelijke acceptatie van besluiten. Indirect kan het bovendien bijdragen aan een vergroting van het maatschappelijke sociaal en menselijk kapitaal, respectievelijk door netwerkvorming en door het delen van kennis. Hoewel op overheidsniveau het grote publiek en patiëntenorganisaties af en toe betrokken worden bij besluitvorming over bepaalde beleidsontwikkelingen binnen de biomedische wetenschap, zoals rondom kloneren, xenotransplantatie en onderzoek aan menselijke embryo's, lijkt een eerste literatuurscan erop te wijzen dat patiënten zelden betrokken worden bij besluitvorming over concrete onderzoeksagenda's van instituten of individuele onderzoekers.

In het licht van deze discussie is de **centrale onderzoeksvraag** in dit proefschrift:

> *"In welke mate is effectieve patiëntenparticipatie in besluitvorming over biomedische onderzoeksagenda's mogelijk?"*

Om deze vraag te kunnen beantwoorden zijn de volgende drie deelvragen geformuleerd:

> *I. Wat veroorzaakt de schijnbare beperkte participatie van patiënten in besluitvorming rondom biomedische onderzoeks-agenda's?*

II. *Welke strategie kan ontwikkeld worden om patiënten actiever en effectiever te betrekken bij deze besluitvorming?*

III. *Wat kunnen we leren van de praktische implementatie van deze strategie, in het bijzonder wat betreft haar effectiviteit?*

Deze drie deelvragen bepalen min of meer de structuur van het aan dit proefschrift ten grondslag liggende kwalitatieve onderzoek. Het eerste deel van het onderzoek bestaat uit beschrijvend-analytisch onderzoek dat focust op de huidige situatie wat betreft patiëntenparticipatie in besluitvorming over biomedisch onderzoek. Klopt de aanname dat patiënten slechts beperkt participeren in deze besluitvorming met de werkelijke situatie? En als dat zo is, wie of wat zijn daar dan de oorzaken van? Het tweede deel van het onderzoek is meer conceptueel van aard en omvat het ontwerpen van een participatiestrategie die effectieve patiëntenparticipatie in besluitvorming rondom biomedisch onderzoek mogelijk zou moeten maken. Hierbij wordt uitgegaan van de analyse en interpretatie van eerdere bevindingen zowel binnen als buiten het kader van dit onderzoek, aangevuld met literatuuronderzoek. Het derde deel van het onderzoek bestaat uit een 'maatschappelijk experiment' waarin de geformuleerde strategie wordt toegepast en geëvalueerd in een concrete situatie. Het primaire **doel** van het hele onderzoek is het reflecteren op, en experimenteren met, participatiestrategieën om daarmee bij te dragen aan een groter inzicht in hoe een geschikte strategie voor effectieve patiëntenparticipatie vormgegeven zou kunnen worden.

In **hoofdstuk 2** wordt de eerste deelvraag geadresseerd. Door middel van een workshop, interviews met allerlei betrokkenen en aanvullend literatuuronderzoek is het huidige Nederlandse *'biomedische besluitvormingsnetwerk'* in kaart gebracht en is gezocht naar voorbeelden van patiëntenparticipatie in besluitvorming over biomedisch onderzoek. Uit de resultaten blijkt inderdaad dat patiënten op dit moment niet structureel deel uitmaken van het biomedische besluitvormingsnetwerk maar dat er wel enkele initiatieven genomen worden om patiënten wat meer te betrekken bij deze besluitvorming. De enkele gevonden initiatieven zijn echter geen effectieve participatie te vertegenwoordigen in de zin van dat patiënten op een directe en gelijkwaardige wijze al in een vroeg stadium in besluitvormingsprocessen participeren en dat hun daadwerkelijke invloed op de uiteindelijke besluitvorming gewaarborgd wordt. Op dit moment worden patiëntenparticipatieinitiatieven veelal vormgegeven als een *ad hoc consultatie* van patiënten of patiëntenorganisaties – al of niet door hen

afgedwongen – of als een zitting nemen van patiëntleden in *gemengde adviescommissies*. Bij de consultatie van patiënten blijft het meestal onzeker of de inbreng van patiënten ook daadwerkelijk onderzoeksagenda's zal beïnvloeden. Maar ook bij de zitting van patiënten in commissies is de beïnvloeding door patiënten marginaal of op z'n best onduidelijk. Ten eerste wordt de precieze inbreng van patiënten zelden expliciet gemaakt, wat het moeilijk maakt deze bewust te integreren in een eindbeslissing of –advies. Ten tweede zijn de posities van patiënten en professionals in dergelijke commissies volgens insiders vaak niet gelijkwaardig en raakt een eventuele patiënteninbreng gemakkelijk ondergesneeuwd door de inbreng van professionals. Hoewel dit deel van het onderzoek zich beperkt heeft tot de Nederlandse situatie, hebben informele gesprekken met relevante actoren uit andere landen en aanvullend literatuuronderzoek laten zien dat de resultaten breder toepasbaar zijn.

Vervolgens zijn, tevens door middel van interviews, de *obstakels* die effectieve patiëntenparticipatie tegengaan, geïnventariseerd en in kaart gebracht. Een groot deel van de beschreven obstakels lijken deel uit te maken van een algemene *'weerbarstigheid'* van het huidige biomedische besluitvormingsnetwerk. Bestaande onderzoeksstructuren, gangbare praktijken en heersende denkwijzen bieden weinig ruimte voor verandering, noch voor andersoortige programma's of projecten noch voor inbreng van patiënten.

Een sterk belemmerende heersende denkwijze, zowel binnen het biomedische onderzoeksveld als binnen de patiëntenwereld, is bijvoorbeeld dat patiënten zich niet met biomedisch onderzoek moeten bemoeien. Ten eerste wordt de biomedische wetenschap door velen beschouwd als een 'esoterische' wetenschap die door de maatschappij met rust gelaten moet worden. Daarbij wijst men op het gevaar dat als het biomedische onderzoek zich teveel gaat richten op maatschappelijke behoeften, dit ten koste gaat van essentieel fundamenteel onderzoek, waardoor de biomedische kennisbasis te smal zou worden. Bovendien wordt benadrukt dat veel grote biomedische doorbraken ontstaan zijn door toeval of vanuit puur wetenschappelijke interesses. Ten tweede worden patiënten niet in staat geacht om mee te beslissen over biomedische onderzoeksagenda's. Besluitvorming over biomedisch onderzoek zou specialistische kennis vereisen die patiënten niet hebben; de ervaringskennis van patiënten wordt zelden als mogelijke waardevolle inbreng voor biomedisch onderzoek erkend. Bovendien zouden patiënten niet in staat zijn over hun eigen subjectieve problemen heen te kijken en

alleen behoefte hebben aan korte termijn sociaal-wetenschappelijk of zorggerelateerd onderzoek.

Betreffende het eerste tegenargument – het niet mogen interfereren in een esoterische wetenschap – is veel geschreven in de literatuur over publieks- en stakeholderparticipatie in besluitvorming aangaande wetenschappelijk(e) onderzoek(sagenda's). Het volstaat hier te benadrukken dat eerder genoemde argumenten die pleiten voor een maatschappelijke beïnvloeding van biomedische onderzoeksagenda's niet inhouden dat al het bestaande biomedische onderzoek maatschappelijk gestuurd moet worden. Er zal een balans gevonden moeten worden zodat er altijd ruimte zal blijven voor fundamenteel, puur wetenschappelijk geïnspireerd onderzoek.

Het tweede tegenargument, dat focust op het (on)vermogen van patiënten om effectief mee te beslissen over biomedisch onderzoek, vraagt om nader onderzoek. In **hoofdstuk 3** wordt daarom de *ervaringskennis* van patiënten en haar mogelijke nut voor biomedisch onderzoek nader onderzocht. Door middel van literatuuronderzoek en aanvullende interviews is de in het vorige hoofdstuk al gestarte inventarisatie van voorbeelden van patiëntenbetrokkenheid bij besluitvorming over biomedisch onderzoek voortgezet. Uit de 22 gevonden voorbeelden blijkt dat de inbreng van patiënten in deze besluitvorming vooral bestaat uit het uiten van *behoeften* aan onderzoeksrichtingen, *ideeën* voor onderzoeksvragen en hypotheses en *oordelen* over de relevantie van onderzoeksprioriteiten of -projecten. Deze behoeften, ideeën en oordelen zijn mogelijke uitingsvormen van de ervaringskennis van patiënten binnen de context van biomedisch onderzoek. Binnen de gevonden voorbeelden is vervolgens gezocht naar casussen die meer inzicht bieden in de concrete, inhoudelijke inbreng van patiënten en dus in hun gebruikte kennis. Slechts enkele voorbeelden bleken dit inzicht te bieden. Al zijn deze casussen mogelijk niet overtuigend genoeg om twijfelende onderzoekers over de streep te trekken, ze zijn in ieder geval een aanwijzing voor het nut van ervaringskennis voor biomedisch onderzoek.

Het doorbreken van de eerder genoemde weerbarstigheid van het biomedische besluitvormingsnetwerk met haar gangbare wijzen van denken en handelen, vergt meer dan een discussie over het mogelijke nut van de ervaringskennis van patiënten. Er is een cultuur- en structuuromslag nodig; met andere woorden: er moet een *transitie* op gang komen. Volgens de transitieliteratuur kan een dergelijke omslag het beste geïnitieerd worden door maatschappelijke druk, die 'top-down' wordt uitgeoefend, te combineren met succesvolle voorbeeldinitiatieven die het

netwerk 'bottom-up' beïnvloeden. Top-down prikkels voor een transitie van het biomedische besluitvormingsnetwerk richting het structureel betrekken van patiënten worden gevormd door de steeds sterker worden maatschappelijke pleidooien (zowel binnen de overheid als in de literatuur) voor patiëntenparticipatie in besluitvorming over biomedisch onderzoek. Nu zijn er nog bottom-up initiatieven nodig, het liefst van invloedrijke en centrale actoren.

Hoe zou een dergelijk initiatief eruit kunnen zien? In **hoofdstuk 4** wordt een voorstel gedaan voor een *nieuwe strategie* die effectieve patiëntenparticipatie zou kunnen realiseren. Omdat een dergelijke strategie moet garanderen dat patiënten in een vroeg stadium en op een directe wijze in het besluitvormingsproces betrokken worden en dat hun inbreng de uiteindelijke besluitvorming expliciet beïnvloedt, is gekozen voor een aanpak die een expliciete *consultatiefase* combineert met een *samenwerkingsfase.* In de consultatiefase kunnen de patiëntenperspectieven en –prioriteiten expliciet gemaakt worden terwijl in de samenwerkingsfase deze perspectieven en –prioritiiteiten (en dus indirect de ervaringskennis van patiënten) doelbewust geïntegreerd kunnen worden in de uiteindelijke besluitvorming. Zo kunnen de tekortkomingen die de eerder beschreven, hedendaagse strategieën voor patiëntenparticipatie hebben, worden voorkomen. Omdat de integratie in de samenwerkingsfase zowel wetenschappelijke (biomedische) kennis als niet-wetenschappelijke (patiënten-) kennis betreft, kunnen we haar *'transdisciplinair'* noemen. Het begrip transdisciplinariteit verwijst naar een vorm van probleemoplossen of kennisproductie waarin wetenschappelijke kennis en maatschappelijke kennis met elkaar geïntegreerd worden. Omdat kennisintegratie tussen zulke verschillende vormen van kennis een complex en delicaat proces is, vergt het een voorzichtige, goed uitgedachte aanpak. Vanuit de literatuur zijn verschillende condities voor een succesvolle transdisciplinaire aanpak gedestilleerd en met elkaar gecombineerd.

Een eerste conditie is de beschikbaarheid van een systematische aanpak. Gekozen is voor een gefaseerde strategie, gebaseerd op de 'Interactive Learning and Action' benadering; een benadering die door de afdeling Biologie en Samenleving is ontwikkeld om kleinschalige boeren te betrekken bij besluitvorming over de ontwikkeling en implementatie van landbouwtechnologieën. De participatiestrategie omvat na een eerste vooronderzoeksfase, een expliciete consultatiefase en een samenwerkings-fase die resulteren in geïntegreerde perspectieven ten aanzien van onderzoeksagenda's. In daaropvolgende prioriterings- en implementatie-fasen kan een definitief programma of plan van actie opgesteld,

respectievelijk geïmplementeerd worden. Andere condities voor een succesvol transdisciplinair participatieproces betreffen de sociale setting en de kwaliteiten van de procesmanagers die optimale kennisintegratie moeten faciliteren en stimuleren.

In de **hoofdstukken 5 en 6**, tenslotte, wordt de voorgestelde strategie in de praktijk getoetst. De beschreven casus betreft een initiatief tot patiëntenparticipatie in onderzoeksagendering in opdracht van het Nederlands Astma Fonds (NAF) en mede gefinancierd door ZonMw. In dit initiatief werden patiënten, naast wetenschappers en zorgverleners, betrokken bij het opstellen van een 'maatschappelijke onderzoeksagenda' rondom astma en COPD die als basis moest dienen voor te formuleren onderzoeksbeleid en onderzoeksprogramma's.

In hoofdstuk **5** wordt de consultatie van astma- en COPD-patiënten (NAF-leden) apart beschreven. Behalve dat deze deelfase een essentieel onderdeel van het hele onderzoeksagenderingsproces is, biedt het ook de mogelijkheid om in de praktijk te onderzoeken of patiënten inderdaad op basis van hun ervaringskennis in staat zijn om adequaat te participeren in een onderzoeksagendering waar biomedisch onderzoek deel van uit maakt. Daarmee kan de houdbaarheid van het eerder genoemde 'tweede' argument tegen patiëntenparticipatie – namelijk dat patiënten daar niet toe in staat zijn – nader onderzocht worden.

De consultatieprocedure bestond uit twee stappen. Door middel van focusgroepen werden astma/COPD-patiënten in eerste instantie gevraagd naar de ervaren problemen met hun ziekte en naar oorzaken van en argumenten rondom die problemen. Daarmee werd aangesloten bij de directe ervaringen van patiënten en tevens een aanzet gegeven voor het formuleren van te prioriteren onderzoeksdoelen. Vervolgens werd een brede enquête gebruikt om patiënten die onderzoeksdoelen op een expliciete en kwantitatieve wijze te laten prioriteren.

Uit de resultaten blijkt dat de betrokken astma/COPD-patiënten goed in staat zijn om te participeren in een brede onderzoeksagendering en naar alle waarschijnlijkheid ook in een specifiek biomedische onderzoeksagendering. Ten eerste waren ze in staat om een breed scala aan (waaronder biomedische) onderzoeksonderwerpen te prioriteren op een onderbouwde manier. Deze onderbouwing is terug te vinden in de resultaten van de focusgroepen. Ten tweede prioriteerden ze biomedische onderwerpen als hoogste en hadden ze dus niet alleen aandacht voor korte termijn sociaal-wetenschappelijk of zorggerelateerd onderzoek. Ten derde hadden de betrokken patiënten enkele biomedische onderzoeks-onderwerpen geformuleerd en geprioriteerd die in huidige onderzoeks-

programma's niet voorkomen, namelijk onderzoek aan co-morbiditeit en onderzoek aan de bijwerkingen van, en onderlinge wisselwerking tussen, medicijnen. De betrokken astma/COPD-patiënten zijn dus niet alleen in staat om te prioriteren, ze hebben, op basis van hun ervaringskennis, ook iets bij te dragen aan huidige biomedische onderzoeksagenda's.

Aangenomen dat andere patiënten hiertoe ook in staat zullen zijn, is met dit onderzoek het hierboven genoemde tweede argument tegen patiëntenparticipatie ontkracht. Bovendien wordt met de resultaten van hoofdstuk 3 en 5 het inhoudelijke argument vóór patiëntenparticipatie, welke verwijst naar de mogelijke bijdrage van patiënten aan de kwaliteit van de besluitvorming, versterkt.

Hoofdstuk **6** beschrijft het overkoepelende participatieve agenderingsproces, zoals dat conform de in hoofdstuk 4 geformuleerde strategie heeft plaatsgevonden. Het proces heeft geresulteerd in een 'maatschappelijke onderzoeksagenda' van 40 onderzoeksonderwerpen die in 10 verschillende thema's waren ondergebracht en in een globale prioritering van deze onderzoeksagenda.

Vervolgens is de effectiviteit van de bewerkstelligde participatie, in termen van het bereiken van vooraf gedefinieerde en normatief en inhoudelijk geïnspireerde doelen, geëvalueerd aan de hand van een van te voren opgesteld evaluatiekader. Evaluatie van zowel de legitimiteit en de rationaliteit van het proces, als de kwaliteit van directe en indirecte uitkomsten wees uit dat de gevolgde strategie geschikt is voor het effectief betrekken van patiënten in besluitvorming rondom onderzoeksagenda's. Enkele nog suboptimale aspecten betroffen de niet helemaal evenwichtige vertegenwoordiging van stakeholders in de samenwerkings- en prioriteringsfasen en de minimale zichtbaarheid van de patiëntenbijdragen in de uiteindelijke geprioriteerde agenda. Hoewel de gevolgde strategie binnen het beschreven initiatief gebruikt werd voor de agendering van aan astma/COPD gerelateerd gezondheidsonderzoek op zijn breedst, zou dezelfde strategie waarschijnlijk ook goed gebruikt kunnen worden voor het vormgeven van effectieve patiëntenparticipatie in biomedische onderzoeksagendering, aangezien dit laatste op impliciete wijze al deel van het initiatief uitmaakte.

De effectiviteit in termen van daadwerkelijke impact van de patiëntenparticipatie op het uiteindelijke onderzoeksbeleid en de uiteindelijk uitgevoerde onderzoeksprojecten is (nog) niet geëvalueerd. Echter, het feit dat het NAF de geformuleerde onderzoeksagenda momenteel gebruikt als leidraad voor haar verdere onderzoeks-

programmering kan beschouwd worden als een eerste indicatie voor een dergelijk langere termijn effect.

Uit het promotieonderzoek zijn verschillende **conclusies** te trekken. Een eerste conclusie is dat patiëntenparticipatie in biomedische besluitvorming inderdaad nog niet zo veel voorkomt en als het voorkomt het meestal op een ineffectieve en niet-structurele wijze plaatsvindt. Effectieve patiëntenparticipatie wordt tegengehouden door vele obstakels die vooral te maken hebben met de weerbarstigheid van het huidige biomedische besluitvormingsnetwerk. Een tweede conclusie is dat veel patiënten in staat zijn om adequaat te participeren in besluitvorming over biomedische onderzoeksagenda's. Zij zijn zich bewust van het belang van biomedisch onderzoek, zijn in staat om over hun eigen individuele korte termijn problemen heen te kijken, zijn in staat om op een onderbouwde manier onderzoeksonderwerpen te prioriteren en kunnen alternatieve biomedische onderzoeksprioriteiten of –onderwerpen formuleren. Dit laatste versterkt het inhoudelijke argument om patiënten bij besluitvorming over biomedische onderzoeksagenda's te betrekken. Een derde conclusie uit dit onderzoek is dat de voorgestelde, gefaseerde transdisciplinaire strategie, met expliciete consultatie- en samenwerkingsfases, een geschikte strategie is voor het effectief vormgeven van patiëntenparticipatie in besluitvorming rondom biomedisch onderzoek. Het waarborgt zowel een legitiem en rationeel proces als de daadwerkelijke invloed van patiënten op het eindproduct.

Om een dergelijke strategie structureel te kunnen implementeren binnen het huidige besluitvormingsnetwerk zijn nog veel obstakels te overwinnen. Het realiseren van meerdere succesvolle voorbeelden van effectieve patiëntenparticipatie, kan bijdragen aan een verdere transitie van het netwerk. Enkele recente initiatieven tot het toepassen van vergelijkbare strategieën voor participatieve onderzoeksagendering binnen andere contexten zijn daarom erg hoopvol. Verder onderzoek zou zich kunnen richten op het efficiënter maken van de strategie ten behoeve van een succesvolle institutionalisering.

Het onderzoek als geheel heeft bijgedragen aan zowel wetenschappelijke als maatschappelijke doelen. Wetenschappelijk gezien heeft het onderzoek nieuwe inzichten opgeleverd aangaande obstakels en strategieën voor effectieve patiëntenparticipatie in biomedische besluitvorming. Op een indirecte wijze heeft het daarmee bijgedragen aan kennisvorming rondom effectieve stakeholderparticipatiestrategieën binnen de wetenschap in het algemeen. Maatschappelijk gezien heeft het onderzoek bijgedragen aan het opstellen van een maatschappelijke

onderzoeksagenda voor astma/COPD onderzoek, aan het formuleren van een strategie voor effectieve patiëntenparticipatie in biomedische besluitvorming en aan het een stapje dichterbij brengen van een transitie van het biomedische besluitvormingsnetwerk richting een structurele participatie van patiënten.

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